

# In Vivo



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## Corporate Affairs: Biopharma's First Line Of Defense Against Scrutiny

BY WILLIAM LOONEY



Coordinated Open Data Will Drive  
Next-Level Research

MorphoSys: A European Champion  
Crossing The Biopharma Rubicon

The Case for Agile Innovation  
In Health Care

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## Corporate Affairs: Biopharma's First Line Of Defense Against Scrutiny

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ERIC ELENKO

JOEP MUIJERS



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## From The Editor



LUCIE ELLIS

This month *In Vivo* is exploring different issues of prominence for the biopharma industry – with reputation leading the discussion.

Our cover story is focused on the evolving role of a critical but often overlooked function within the pharma model: corporate affairs. In an exclusive interview, Amgen's Judy Gawlik Brown gives an open account of what it takes to lead corporate affairs within a large drug company. Brown took on the role of senior vice president for corporate affairs at Amgen in Spring this year, replacing Ray Jordan, who had held the title since 2012. She is responsible for strategic communications across Amgen, including internal and external comms,

issues management and philanthropy. Previously, Brown worked at Perrigo Company as EVP of business operations and CFO. She makes use of this in her mission to reposition Amgen as a "truly global player."

Brown also discusses the ever-important topic of reputation. "There is no doubt that it is tough to maintain a strong reputation in the context of changing societal expectations," she tells *In Vivo*'s William Looney. "There are companies in other areas who have maintained their reputation when the broader industry reputation suffered." One example, Microsoft. Which has "had some success with this by building trust through best-in-class performance in digital security."

Also included this month is a feature interview with MorphoSys' new CEO Jean-Paul Kress. He discusses the European biotech sector and how it is expected to evolve over the coming few years, as key biotech players continue to mature and reach key milestones. "The US market tends to reward innovation ... It's a virtuous circle which works really well. But it is a mentality as much as anything," Kress says, when assessing the European biotech market in comparison.

Elsewhere, Ashley Yeo speaks with executives at PureTech Health, a company which has had its own issues with reputation recently. Senior executives Eric Elenko and Joep Muijers reveal more about the focus and aims of the Boston-based group for its biopharma and technology portfolios.

Finally, an editorial note: eagle-eyed readers may have spotted an error in last month's Personalized Medicine Infographic. The product Tegsedi (inotersen) was mislabeled. The company listed for this product should have been Akcea Therapeutics.

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# Up-Front

## SNAPSHOTS FROM OCTOBER'S CONTENT

“Today’s corporate affairs professional role is harder than ever before. Deciding what kinds of content will be most impactful to various audiences and how it should be deployed are important strategic decisions. There are so many options for people. Content of all kinds – from around the world – is literally at their fingertips.”

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– Judy Gawlik Brown,  
Amgen’s SVP For Corporate Affairs

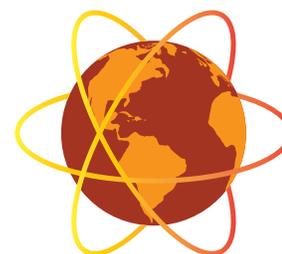


**Akili leads the way in digital medicine and solving cognition problems.** Depression, cognitive issues and MS, are potential applications, after ADHD. PureTech, which owns 34.9% of the company, sees this as a huge unmet need where there are not many pharmacological options.

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One of the most remarkable things about **MorphoSys**, when considering a biotech of its size, is the breadth and depth of its pipeline. It has more than 100 distinct molecules in its pipeline, giving it what it calls “**one of the broadest pipelines in the biotechnology industry.**”

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“ELIXIR is the only life science data infrastructure at this scale offering openly available platforms, tools and services for scientists. So, no matter where you are on the planet, you can access many of those resources and innovate.”

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– **Katharina Lauer**,  
ELIXIR's Industry Officer

### Most Companies Struggle With Four Main Obstacles: PAGE 30



Misaligned funding model for agile



Lack of an enterprise-wide strategy for agile



Company culture that does not support agile



Missing key IT enablers for agile software teams

# ■ Around The Industry

## Can Novartis Reclaim Pioneering Role In Transplantation?

Novartis is developing a new type of immunosuppressive agent, an anti-CD40 monoclonal antibody, which might allow organ transplants to last longer in recipients, reducing the pressure on transplant waiting lists and improving the long-term outlook for patients.

Novartis AG might soon be able to reclaim some of the kudos it once received for developing groundbreaking transplant-enabling therapies like ciclosporin, through its pioneering of a new class of potential products now in early clinical development.

The Basel, Switzerland-based multinational has an impressive heritage in the organ transplant sector going back more than 30 years, but the company has been rather quiet in this therapeutic arena in recent years. However, it is now developing a fully human anti-CD40 targeting monoclonal antibody, iscalimab (CFZ533), which acts to inhibit the CD40 signaling pathway and inhibit activation of CD40-expressing cells such as dendritic cells, monocytes and B-cells.

Only a handful of companies have expressed an interest in this immunosuppressive process – in contrast to what appears to be a greater push to exploit the opposite effect, CD40 activation, for the development of new anticancer agents.

That said, Novartis's iscalimab has achieved remarkable results in early-stage clinical studies that have researchers in the field talking excitedly about the drug's potential role in patients, particularly for prolonging the life of transplanted organs.

Prolonging the durability of organ transplants could be key to reducing the growing demand for donated organs. "Less than 50% of patients who receive a kidney from a deceased donor have that kidney survive more than 10 years," explained Eric Hughes, Novartis's global development unit head for immunology, hepatology and dermatology. After the

donated kidney fails, patients are placed on hemodialysis and/or are added to transplant waiting lists, which for kidneys in the US has more than 100,000 people on it. "Novartis did a great job, 35 years ago, making organ transplants

attack by the patient's immune system, leading to inflammation, the development of scar tissue and, eventually, failure of the transplant.

Standard therapies for immunosuppression like calcineurin inhibitors (tacrolimus, ciclosporin), are associated with a range of side effects, so low doses are administered that appear to allow a patient's immune system to continue to attack organ transplants: "We need



"Everyone is excited about iscalimab. We want to help. Novartis is happy to be contributing to a field we have been involved in for so long,"

ERIC HUGHES

possible. But we need to do better now because the demand for kidneys is so high and the life of a person on hemodialysis is so miserable," Hughes pointed out in a recent interview with *In Vivo*.

"The general public fails to realize the importance of keeping patients off hemodialysis. The procedure has a huge impact on the life of patients, who have to attend a clinic three times a week for three to four hours, and afterwards the patients don't feel great," Hughes said.

But why are kidney transplants not lasting for longer with the benefit of immunosuppressant therapy? It all comes down to achieving a balance between suppressing the immune system and not causing too many side effects; achieving this balance can leave kidneys still under

to find new drugs that prevent the slow inflammatory processes that damage donor kidneys, without the harmful effects of current treatments," urged the Novartis executive.

Researchers are therefore hopeful that anti-CD40 monoclonal antibodies could be those sought-for well-tolerated immunosuppressive agents, with Hughes pointing out that in early studies, "iscalimab is showing great potential."

Novartis is not alone in this endeavor – a small number of pharmaceutical companies are involved in developing anti-CD40 antibodies for improving the durability of transplants. For example, Gaithersburg, MD-based Viela Bio Inc. has VIB4920, an anti-CD40L-Tn3 fusion protein, at the Phase II-ready stage. And Astellas Pharma

Inc. has bleselumab (ASKP1240), an anti-CD40 Mab, in Phase II for the recurrence of focal segmental glomerulosclerosis in de novo kidney transplant recipients, which it is developing in partnership with Kyowa Hakko Kirin Co. Ltd. (Also see "Antibodies emerge as key focus for Kyowa Kirin" - *Scrip*, 5 Jun, 2009.)

But Novartis appears to be leading the anti-CD40 effort, and Hughes said iscalimab had been engineered to potentially be the best-in-class. Any progress would be a nice boost for a company which no longer receives blockbuster sales for its marketed immunosuppressive agents: Neoral/Sandimmune (cyclosporin), had sales of \$110m in the first half of 2019, and Zortress/Certican (everolimus) had sales of \$250m (up by 7%) in the same period.

#### CLINICAL DATA

In presentations at US and EU scientific meetings over this summer, clinical researchers and Novartis researchers reported biopsy data from a small subset of patients from a clinical study of iscalimab versus the calcineurin inhibitor tacroli-

mus, each combined with a treatment backbone of steroids plus mycophenolate. Pathologists found that three of five patients treated with iscalimab had a normal renal histology in their donated kidney, while none of the seven patients treated with tacrolimus had such pristine donated kidneys.

The findings were "very surprising, you wouldn't expect that after a kidney transplant, you always have some level of smoldering inflammation," Hughes said. And other preliminary data suggested that iscalimab was as effective as tacrolimus in protecting transplanted organs from rejection during the first year of therapy. The findings "suggested to us that the drug has the potential to extend the life of transplanted kidneys, perhaps for longer than 10 years," Hughes noted. "We weren't expecting the strength of the response or the type of response," he added.

With all immunosuppressive drugs you run the risk of infections, but the data right now on the side effects of iscalimab were encouraging, Hughes continued. In preclinical studies, iscalimab did not

increase the rate of Epstein-Barr virus infection, and was well tolerated.

Phase IIb studies have already started with iscalimab, and are enrolling faster than scheduled. The company is hoping that it can accelerate development of the drug in discussions with health authorities.

Iscalimab also has promise in other auto-immune conditions. "Top of our list right now is Sjögren's disease, another condition that has no good treatments, and where patients are fatigued to such an extent that it affects their daily lives," Hughes said. The company is also evaluating the product in liver transplant patients, and one day hopes to extend those activities to include cardiac transplant patients.

As well as improving the lot for patients, the development of iscalimab is having a beneficial effect on the mood in Novartis. "Everyone is excited about iscalimab. We want to help. Novartis is happy to be contributing to a field we have been involved in for so long," Hughes concluded.

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JOHN DAVIS

## International IP Arbitration – A Blessing Or A Bad Idea?

Medtech companies traditionally resolved disputes over IP rights before national courts, but more and more of them are now turning to international arbitration to protect their IP rights. Dorothee Schramm, a partner at Sidley Austin specializing in international commercial disputes, explains the benefits of this growing trend and the pitfalls to avoid.

Intellectual property (IP) has an enormous value in today's global innovation-driven economy. It is also relevant to common medtech contracts, such as collaboration agreements for R&D, production and marketing, licensing and distribution agreements, as well as technology transfer agreements.

Such contracts with foreign partners typically contain arbitration clauses to resolve disputes that may arise and that cannot be resolved through negotiations and/or mediation. (Also see "Conflict Management Strategies And Dispute Resolution Clauses – Ensuring Your In-

ternational Contract Will Be Enforced" - *In Vivo*, 4 Feb, 2019.)

#### PATENT DISPUTES IN A JOINT R&D PROJECT

Arbitration clauses can have an important impact on IP rights. Many companies collaborate and contribute to joint R&D projects. Agreements for such projects typically provide for joint patent ownership if several partners contribute to a joint invention, and/or for perpetual, royalty-free access rights to all knowledge resulting from the project. There is always the possibility following the completion of

the joint R&D project, that project results make their way into patent applications of some partners and into the products of other partners.

If the partner seeking the patents then sues another partner for patent infringement, the infringement suits can be brought in several jurisdictions, depending on where the products are sold and manufactured. In such a scenario, an arbitration clause in the joint R&D agreement may allow the partner who is sued for patent infringement to initiate arbitration in a neutral forum to claim co-ownership and access rights to the asserted patents, withdrawal of the infringement suits and the cost of legal fees incurred in defending those suits as damages.

In this exemplary scenario, international arbitration can help a company enforce its IP rights and avoid legal battles

that may take years, involve significant legal costs and risk conflicting decisions by different national courts in different countries. This scenario is typical in that most arbitrations over IP are about disputes over patents. Such disputes often relate to:

- Patent ownership, including transfer of patents: examples are joint R&D cases, and cases of improvements patents conceived during a contractual cooperation;

- Utilization rights to patents versus patent infringement: examples are joint R&D cases, and licensing agreements where the licensee (allegedly) uses the licensed patent in new or modified products beyond the limits of a license or after termination of a license; and

- Patent invalidity: examples are cases where the licensor argues that it owes no licensing fees because the patent is invalid, and cases where patent invalidity is invoked as a defense against allegations of patent infringement.

### UPS AND DOWNS OF TRADITIONAL CARVE-OUT CLAUSES FOR IP DISPUTES

Traditionally, many contracts with arbitration clauses contained a carve-out for IP disputes to be resolved by national courts rather than by international arbitration. Such carve-outs are becoming less frequent, as parties are favoring arbitration for all disputes.

The main reason for traditional IP carve-outs was the limited effectiveness of arbitration with regard to patent ownership and patent invalidity actions. Indeed, one must be aware of arbitration's limitations to properly address these issues. Courts have the advantage that their decisions on patent ownership and patent invalidity can be directly registered in the national patent register. In arbitration, only some liberal countries (such as Switzerland) allow the holding of an arbitral award to be directly registered in the patent register. In most other countries (for example in France), arbitral tribunals can decide disputes about patent ownership or patent invalidity, but the findings will be effective only as between the parties and will not be directly entered into the patent register.



If a dispute concerns patent invalidity, obtaining an award that affects only the rights between the parties is almost always sufficient to satisfy the claimant's main interest, such as a licensee's interest in having the licensor's claim for royalty payments or patent infringement dismissed. In the pharma industry, the limited effect of a confidential award finding a patent to be invalid can even be advantageous for both parties, as it may clarify the situation between them without allowing competitors to benefit from the patent invalidity. The situation is different if the dispute concerns patent ownership. In such cases, your contract can provide for a contractual right to have patent (co-)ownership transferred, which should in most instances be enforceable the same way as, for example, a claim to have real estate ownership in a land register transferred. In any case, a well-reasoned award may have a deterrent effect on the unsuccessful party not to risk proceedings before a patent court.

The joint R&D example illustrates the downside of traditional carve-out clauses. In the case of a carve-out, claims for transfer, invalidity or infringement of patents must generally be brought in each country where the patent is registered, due to the territoriality of patents. In some countries, a patent invalidity defense against an infringement action must be brought before yet another court (for example in Germany, where an invalidity defense must be brought before the Federal Patent Court, leading to parallel proceedings). The same commercial dispute may end up before several courts in different countries.

Moreover, many commercial disputes concern a variety of issues, some of which may be IP related while others may not. In such cases, a carve-out clause may not only split a commercial dispute between different jurisdictions, but may also result in disagreements about whether certain aspects of the dispute must be submitted to arbitration or state court litigation. Carve-out clauses therefore often lead to costly disputes over jurisdiction in addition to multiple parallel proceedings, which are costly, difficult to coordinate, often involve several legal teams and may lead to contradictory outcomes.

These downsides have contributed to the rise of IP arbitration, which can offer advantages in the form of six Cs: consolidation, confidentiality, choice, capability, clock and cost.

As the joint R&D example described above illustrates, international arbitration offers the possibility to consolidate the overall dispute in one arbitration. Another important advantage of arbitration is the confidentiality of the arbitration proceedings, provided that the contract provides for a seat of arbitration or arbitration rules that provide for confidentiality. An arbitration hearing involves only the parties, whereas a court hearing in infringement or other proceedings often includes the general public (including potential competitors).

Arbitration also offers the parties the choice of the decision makers, which often leads to panels of arbitrators with different areas of expertise. Furthermore, arbitration offers a better chance to properly present one's case, and is capable of delivering more solid results. With skilled arbitration counsel, even the limited document production typically granted in arbitration often leads to the production of documents that have an important impact on the outcome of the case. Moreover, in IP cases involving scientific questions, arbitral tribunals often develop a much deeper technical understanding on a broader evidentiary basis as compared to a patent infringement court.

Another important aspect is the time required to resolve patent disputes. In the joint R&D example, a final arbitration award could be rendered while the parallel infringement proceedings are still ongoing. This is confirmed by a working group

## 5 TIPS FOR BRINGING IP DISPUTES TO INTERNATIONAL ARBITRATION



**1.** Carefully choose the place of arbitration and arbitral institution. It is safest to choose one of the major and most reputable places and institutions. Aspects which are relevant to this choice include how liberal the place of arbitration is regarding IP arbitration, the confidentiality of the arbitration proceedings, and the availability of effective interim relief.

**2.** Make the arbitration clause sufficiently broad. To cover patent infringement claims in connection with a contract dispute, phrase the arbitration clause appropriately. For example, you can refer “all disputes arising out of and in connection with the contract, including tort and IP infringement claims” to arbitration

**3.** Clarify the contractual IP rights, especially in joint R&D projects. And specify the contractual rights of the parties and of their (present and future) affiliates to patent (co-)ownership and access rights. You should also contractually define terms that may have different meanings under different patent laws – such as the term “contribution to an invention” entitling a party to patent co-ownership under the contract, as different patent laws pose different requirements for such contribution. Finally, consider drawing up a list of background knowledge and background patents, and addressing improvements to background knowledge and patents.

**4.** Clarify the contractual remedies. Include language that will allow an arbitral tribunal to make orders to achieve full effectiveness of the award between the parties, for example orders against one party to give up or (partially) transfer patents. It is also worth clarifying that a contractual right to patent (co-)ownership entails the (royalty-free) right to use and sublicense the patent, as this right will stand even if there are problems with the patent transfer.

**5.** Be careful with the requests for relief. Phrase the relief you are seeking with care and include alternative requests. This can minimize the risk that the remedies you seek with regard to registered patents go beyond those that are recognized by the law at the place of arbitration and/or the place of enforcement, in particular the place of the patent registration.

on life sciences dispute resolution appointed by the Arbitration and Mediation Center of the World Intellectual Property Organization (WIPO), which recently published figures for the average duration of patent litigation in selected countries. In those countries (including the UK, US, Japan, China, Brazil, India, Russia, Republic of Korea and several EU countries), the duration of all instances exceed the typical duration of an arbitration, often significantly. While an arbitration often costs more than a court proceeding, parties can potentially obtain full compensation for legal fees, which is not the case before many courts. Moreover, it is often cheaper to have one arbitration than several parallel court proceedings.

In many IP disputes, the availability of interim relief is also important, for example for avoiding the transfer of a disputed patent in an entitlement dispute, or for preventing a patent infringer from

entering into or staying on the market. If a contract provides for arbitration at an internationally reputable seat, parties will typically have the choice between requesting interim relief from an appropriate national court or from the arbitral tribunal. Moreover, the arbitration rules of the world’s most reputable arbitral institutions provide for the possibility of appointing an emergency arbitrator if urgent interim relief is required before an arbitration has even started.

While IP arbitration has many benefits, it is also important to understand the risks. The flip-side of consolidating different court proceedings into one arbitration is that the parties put all their eggs in one basket. As a consequence, if a company brings an IP dispute to arbitration it better do it well. This is particularly the case because IP arbitrations can be complex since diverse national patent laws may apply to the invalidity

of a patent in different countries. That said, a company would have to tackle the same laws anyway if there were multiple court proceedings in different countries, and it can better streamline the work in arbitration as it involves only one legal team. As both parties will face this same challenge, they can also agree on the application of one or several “proxy” patent laws to simplify matters.

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DOROTHEE SCHRAMM

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# Corporate Affairs: Biopharma's First Line Of Defense Against Scrutiny

BY WILLIAM LOONEY

The corporate affairs function is biopharma's first line of defense against today's business culture of relentless public scrutiny, a trend compounded by the frequently preemptive viral judgments of social media.

In Vivo talks to Judy Gawlik Brown, Amgen Inc. new senior vice president responsible for corporate affairs, on how her team is contributing to the biotech leader's drive to update its image as a truly global player in both product and process innovation, combining leading-edge manufacturing efficiencies with a growing pipeline of novel precision medicines for patients with unmet medical needs.

So what? Amgen is a bit of a paradox, being well-known chiefly for its low public profile. A corporate rebranding effort now underway focuses on a bigger global footprint, its historic culture as a biotech in a big pharma world, and an active role shaping future health budgeting and innovation policies, particularly in the US.



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## AMGEN'S JUDY GAWLIK BROWN IS USING HER FINANCE AND CONSUMER HEALTH BACKGROUND TO MAKE THE BIG BIOTECH'S MESSAGE MORE AUTHENTIC AND ACCESSIBLE – A BRISK RESET AGAINST TODAY'S DISCONNECTED WORLD OF ALTERNATIVE FACTS.

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**S**enior VP for corporate affairs at Amgen, Judy Gawlik Brown, aims to change how the big biotech curates and distributes messaging for both internal and external audiences. It involves a redeployment of media channels like websites, social listening, data analytics and introducing employees as company ambassadors in multiple markets. The new thrust is expressly global – with content that reinforces CEO Bob Bradway's strategy to expand Amgen's commercial presence outside the US.

In addition to being multi-lingual, Brown is unusual in bringing a lengthy finance background to the corporate affairs job. And finance is a sector that has much to teach pharma on building a more consumer-friendly image. Brown promises to introduce hard metrics of performance in tracking the company's plans to compete in a changing health policy climate marked by transparency, evidence and patient-centric engagement.

In an exclusive interview, Brown told *In Vivo*, that the biggest single challenge facing biotech is a closing door on health financing. Failure to build a broad, multi-stakeholder consensus on budgeting for tomorrow's health needs – a goal that remains elusive – could squander future drug development opportunities from large population-health data sets like Amgen's deCODE genetics venture, as well as other advanced technology tools to cure and treat disease.

***In Vivo:*** Your professional background is notable for its diversity and breadth – in pursuit of that career north star, you have bent the light in virtually every direction. What circumstances brought you to Amgen and how do your prior assignments inform your latest role, as the company's corporate affairs lead?

**Judy Gawlik Brown:** I grew up in the suburbs of Chicago, studied accounting as an undergraduate at the University of Illinois at Urbana-Champaign and later received my MBA at the University of Chicago. Accounting seemed like a very practical – and appropriately Midwestern – course of study but there was another side to me too. In high school, I studied ballet and, through that, discovered a love of the French language. This led to an interest in living and working abroad, which I was able to do thanks to the geographic versatility of my accounting background.

EY was my first employer. Not long after discovering how quickly I learned French, they offered me an opportunity to relocate to Germany. "I've heard you have a facility with languages," said my manager at the time. "I'm sure you'll figure out German quickly." That's when I realized a basic fact of corporate life: when you have a reputation for something, the pressure is on to live up to it.

I later held operational and brand management roles at



*“I was struck by the fact that everyone who works here is given license to think about our products and technologies in new ways.”*

**JUDY GAWLIK BROWN,  
AMGEN INC. NEW SENIOR VICE  
PRESIDENT RESPONSIBLE FOR  
CORPORATE AFFAIRS**

Whirlpool’s European headquarters in Italy and the Michigan corporate offices. This was followed by a nearly 11-year stint as executive VP and chief financial officer at the consumer health care and pharmaceutical company Perrigo Co. PLC, where I managed groups that included finance, IT, investor relations, corporate communications and government affairs. This allowed me to bring a diverse set of experiences to my present role at Amgen.

**You obviously see language skills as a window on management and cultural awareness. How does that play out in your management approach and style?**

Learning a new language in a foreign country brings you closer to people in a fundamental way. It also helps you exercise your mind on unfamiliar turf. When I travel on business, I make a point of picking up some basic conversational skills. I am convinced that, by doing this, I can meet people more than halfway in building relationships of mutual trust. I’m letting local managers know I am here for them, not the other way around. I’m convincing them of my desire to engage culturally while conversing in words, not just numbers. I recently went to Japan to meet with our team there. Building out our global presence is an important strategic objective for Amgen so building relationships across geographies and cultures comes with the territory.

**Besides accounting and audit consulting, you have served in numerous operational roles, spanning the appliance manufacturing, marketing and OTC drugs businesses. What drew you to venture into biotech by joining Amgen in 2017 as SVP of global business solutions and finance?**

In my position at Perrigo, I pursued practices that helped consumers save 30% to 40% on popular over-the-counter medicines, which helped lower the cost of treating common chronic diseases. I liked the societal consequences of making the business more efficient and passing some of the savings on to consumers.

What motivated me about Amgen, besides its stature as the one of the world’s largest biotech enterprises, is its unequivocal focus on helping the patient. The company’s mission is remarkably simple: to bring innovative medicines to seriously ill patients where there is large unmet medical need. Cancer. Cardiovascular disease. Osteoporosis. It came up in every conversation I had during my onboarding visits at the Thousand Oaks HQ campus in southern California.

The culture is also incredibly alluring. While the company has a long tradition of consistent year-over-year financial growth, it still functions like a start-up. Ideas are encouraged to come from anywhere. When I started, the company was deep into a years-long transformation initiative that included reinventing business processes and technologies to secure better outcomes for our patients and commercial partners alike. I was struck by the fact that everyone who works here is given license to think about our products and technologies in new ways.

**Does that existential interest in serving patients extend beyond the US home base to the approximately 100 countries where Amgen has a presence?**

Our messaging about the patient as the focal point in health care is universal, but naturally there must be some tailoring because of local country rules on how the industry can interact with patients and health professionals.

**Now that you direct the corporate affairs portfolio at Amgen, what do you see as different in managing this assignment today versus five or 10 years ago?**

During my time at Perrigo, I created corporate communications and government affairs functions in my organization. I advocated for this investment because of Perrigo’s strong growth trajectory at the time, the changing corporate tax strategic landscape, the importance of consumer/patient liaison work in support of our product offerings, and other stakeholder outreach efforts related to an increasingly global footprint made possible by dozens of M&A transactions. While this gave me some experience for my present role, managing corporate affairs today

is a complicated assignment, particularly in today's complex business environment. When it comes to the basic task of distributing and exchanging information, the way you did that was a given for 20 years. The institutional structure was essentially fixed. No longer. Now information is unbounded by those norms and traditions. We operate in a global, 24-hour information cycle, drawing from an endless array of technologies and platforms. The media and technology landscapes have changed drastically. There are few barriers to entry. Unfortunately, the separation between fact and opinion has blurred, not to mention the presence of disinformation. Anyone can position themselves as a trusted source.

So, today's corporate affairs professional role is harder than ever before. Deciding what kinds of content will be most impactful to various audiences, be it earned or owned, and how it should be deployed, are important strategic decisions. There are so many options for people. Content of all kinds – from around the world – is literally at their fingertips.

### How do you distinguish between earned and owned media?

As a society, we are experiencing some very different dynamics when it comes to trust in traditional institutions. Some have even gone so far as to call this a “trust crisis.” Broadly speaking, there is some research to suggest that people trust individual CEOs and others in business more than they do traditional media and politicians. So, the messenger matters, and this phenomenon is leading to an adjustment in how we operate. A recent example of earned media involved working with a top-tier US newspaper outlet on a story about the launch of one of our new medicines. We spent almost a year cooperating with the reporters and answering their questions, of course without any assurance on how things would turn out. It was a good story and an outcome that was largely positive for Amgen. But it took a lot of time to play out.

Another approach is to focus on developing similar content ourselves – about products, human capital, leadership, strategy and philanthropic activities – and publishing it on the channels we own, such as our websites and social media channels. Developing a sophisticated corporate affairs capability that integrates both approaches, that is informed by social listening and other data analytics, and that leverages employees as company ambassadors on social media are important areas of focus for our team.

The other important dynamic at play is the fatigue we face as a society with overtly commercial messages related to corporate brands. Creating content that is an authentic expression of the Amgen brand, and that speaks to audiences in a way that goes beyond the traditional advertising model, will be part of our evolution.

### On the topic of reputation, do you believe it is possible for an individual company to shine even though the industry it represents is held in low public regard?

Purpose is the only way forward for business. Increasingly, forward thinking leaders are galvanizing their businesses around purpose to ensure a strong reputation. Our research indicates that our purpose to “dramatically improve people's lives through biotechnology” helps ensure that our 21,000 colleagues at Amgen are excited and motivated to work for us. Reputation is built on actions, and when each and every member of your organization – across the product life-cycle – is engaged with your purpose, great things happen.

There is no doubt that it is tough to maintain a strong reputation in the context of changing societal expectations. There are companies in other industries, however, who have maintained their reputation when the broader industry reputation suffered. Microsoft has had some success with this by building trust through best-in-class performance in digital security. Similarly, PayPal overshadows many brands within the financial services sector as a trusted provider of transaction technologies. We believe we have an opportunity through our distinct advantage



*“We believe we have an opportunity through our distinct advantage of being a biotech in the pharma industry.”*

**JUDY GAWLIK BROWN,**  
AMGEN INC. NEW SENIOR VICE  
PRESIDENT RESPONSIBLE FOR  
CORPORATE AFFAIRS

of being a biotech in the pharma industry, through our focus on scientific leadership, and through our patient-centric purpose that is backed up by our performance.

**How are corporate affairs activities deployed within the Amgen HQ organization?**

I am responsible for all corporate communications, internal and external, as well as philanthropy and social responsibility efforts, including our charitable efforts to inspire the next generation of scientists, and providing Amgen medicines at no cost to eligible low-income patients.

**What is your strategy for this first year as Amgen’s corporate affairs lead? Would you describe your mandate as one of incremental progress or a wholesale remake?**

I am not a fan of incrementalism. I see my charge as an opportunity to test new approaches consistent with a single question: what activities within our purview have the most potential to add value and move the needle forward for Amgen’s business and reputation, building on the resources we already have on hand? Every day, through the work of corporate affairs, we are actively introducing Amgen to more people with messages that will resonate globally in line with management’s strategy to grow our presence outside the US.

In June, I announced a restructuring of the corporate affairs team. It was a redeployment, not a retrenchment. By that I mean we identified some areas where we were under-investing and moved resources around to fill the gap. The most important gap was in content creation.

The result is that we are pursuing a more expansive, rigorous, and carefully targeted approach to the content we create at Amgen. The idea is to tell the company’s story better, add more coverage of what we do for patients, health care and society in markets outside the US, emphasize the successes that stem from our partnerships, and break down what the great science we conduct in the lab will do in tackling diseases in major areas of unmet need.

It is also important to extend the discussion of what constitutes innovation at Amgen. There is a tendency to end the conversation on innovation at the lab or in the product line when there is a wealth of ideas bearing fruit in manufacturing, or in the progress our supply chain operations team has made to ensure that no Amgen patient misses a day of medicine, even in the midst of fires and hurricanes. Amgen is an industry leader at leveraging huge, population-level genetic data sets to drive drug development. Finally, there is innovation taking place in how we talk to patients in the digital space. We are doing a lot of strategic storytelling there, which includes having two-way conversations with patients and health care providers on social media. When they reach out to us on social media, we now have a process in place to rapidly respond as appropriate. We also share our story with and seek input from influential patient bloggers. All these diverse platforms are innovative instruments that allow us to amplify the message. These exist throughout the company, so the idea is why limit Amgen’s public conversation to medicines?

A strong communications mindset delivers results that extend far beyond public relations. When we share these great stories, it draws Amgen closer to the pool of top-notch human capital.

It makes the best people in tough-to-recruit fields like data science and clinical trial logistics want to come to work at Amgen. Our contributions in attracting and retaining talent makes our function strategic. And I intend to show that with metrics that document our performance. As a former CFO, I certainly cannot say that metrics in corporate affairs do not matter.

**You have a new Amgen Responsibility Highlights Report coming up in 2020. What direction will this important marker of corporate reputation take under your leadership?**

It is clear to us that investors, future employees and other stakeholders want to learn more about the Environmental, Social and Governance (ESG) performance of companies like Amgen. As a result, we are shaping our approach to corporate responsibility to focus on broad areas that are both relevant to our business and important to society – these include access to medicines, ethics, environmental sustainability, and social investments via our philanthropy efforts. Next year’s Responsibility Highlights Report will be a continuation of reporting on progress across all these areas, and how our mission as a company – to serve patients – goes beyond making life-saving medicines. Further, we are striving to issue this kind of communication more regularly, and in different formats, not just annual reports.

Our report will highlight the many ways in which we contribute to society, with an emphasis on our use of innovative approaches. For instance, our ongoing support for STEM education now includes a partnership with Harvard University to develop LabXchange, a sophisticated education technology platform that will give students studying biology around the world access to a unique virtual lab experience for free, dramatically expanding the Amgen Foundation’s reach in science education.

**Inevitably, ambitions must confront the intrusions of an increasingly disruptive environment – the harsh world of hard things. What keeps you up at night as you build the corporate affairs function at Amgen?**

There is a looming global crisis in health care financing and the demographic transitions in the US, Europe, Japan and even China is only one aspect. When we talk about health, I get concerned about the tendency of stakeholders to ignore this big picture problem. We have piecemeal debates focused on dividing up the spending pie, not growing it. The major stakeholders – including our industry – don’t do enough to take the initiative and reach out to other interests to confront the fundamental question: who’s going to pay? Health care is a societal concern. If we don’t do better job of seriously reaching out for consensus solutions to health financing, I fear that the industry, patients and the public will suffer. Science alone is not going to ensure that your mother gets the medicine she needs and stays on it. This is the big issue that, sadly, gets swept aside by today’s 24/7 news cycle. We need a broader coalition of interests to recapture the debate so innovation will continue to transform lives. ❁

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# Meddevicetracker: Medical Device Intelligence and Forecasts

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# Coordinated Open Data Will Drive Next-Level Health Research



Experts from a European data sharing initiative talk to *In Vivo* about the importance of coordinated computational biology efforts for the future of life sciences innovation and the effective use of artificial intelligence.

## BY LUCIE ELLIS

As big data continues to change processes in the life sciences sector, bioinformatics companies are growing in numbers to provide more efficient collection and analysis services.

ELIXIR was created to better coordinate the evolution of bioinformatic services across Europe.

The main aim of ELIXIR is to make the data from EU-funded and internationally funded research projects available for others to use. This includes among others, genomics, proteomics, metabolomics and image data.

**E**LIXIR, founded in December 2013, is a joint effort between several European countries for the development of coordinated, integrated and sustainable public bioinformatics services. “There are many public databases, software tools and training courses that already have extensive usage by academia and the life sciences industry. However, there is lot of fragmentation, so those resources don’t always work closely together,” Andrew Smith, ELIXIR’s head of external relations, told *In Vivo*. The group aims to better coordinate the evolution of bioinformatic services across those member countries.

ELIXIR has connected 22 countries. In each participating country there is a national effort to coordinate the public bioinformatics services. “Overall, across those 22 countries that are involved, we have 220 institutes. This means that we have about 700 scientists that run those services and are involved in the network too,” Smith added.

The main aim of ELIXIR is to make the data from EU-funded and internationally funded research projects available for others to use. This includes amongst others, genomics, proteomics, metabolomics and image data. “There are lots of new data being produced and we need to make sure that they are coordinated so users in industry and academia can access them. Computational biology itself really helps scientists to address some of the grand challenges; it helps companies to be able to develop new drugs, food agriculture companies to develop new drought-tolerant crops and so on. It is at the heart of a lot of research tactics gracing the life sciences,” Smith highlighted. “We find that these public resources are used extensively by industry and we want to further improve the offering.”

## Coordinating Research

Interoperability is a huge challenge for the life sciences sector in Europe. “There are hundreds of different databases, describing different data sets and they don’t always use the same ontologies,” Smith said. Different standards exist across these sources and although the public offerings are extensively used by companies, the greatest value is achieved when these resources can talk to each other. Smith added: “Right now, there is still a lot of fragmentation. For example, how data sets are described by those in industry can vary to how they are described by those in the public domain; or how ontologies used in the clinic are different to those used by research scientists. There is still a lot of work that needs to be done with academia and industry to integrate approaches.”

When a country joins ELIXIR it provides a financial contribution to the organization. This money is used to fund technical projects that help to make connections and secure infrastructure that is more sustainable. ELIXIR has around 50 such projects ongoing. Smith described one example: “There is a resource called European Genome-Phenome Archive, which is run jointly by CRG (ELIXIR Spain) and the European Bioinformatics Institute. It stores a lot of sensitive human data.” The archive is addressing the issue of ethical and legal restraints on transferring personal data across national boundaries. “ELIXIR is helping other countries to connect their own local installation of this database so that the metadata can be transferred and stored in one repository but the sensitive human data, which in many cases can’t leave the national jurisdiction in which it was generated, can stay there,” Smith explained.

## Defining Open Science

“Open science is making scientific research and the dissemination more transparent and accessible,” said Katharina Lauer, industry officer for ELIXIR. “Being open can be seen as the driver for progress and tackling these grand scientific challenges and breakthroughs. ELIXIR is the only life science data infrastructure at this scale offering openly available platforms, tools and services for scientists. So, no matter where you are on

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*“There are hundreds of different databases, describing different data sets and they don’t always use the same ontologies”*

– Andrew Smith

”

the planet, you can access many of those resources and innovate.”

Lauer highlighted that not all data should be made available and be shared. Privacy is a major concern in the health care sector and industry has considerations around proprietary data for commercial reasons. “But in other sectors, especially in the computational sector, there has been a huge move toward working more collaboratively and more openly,” she noted.

ELIXIR is involved in the FAIRplus project, an effort through the Innovative Medicines Initiative (IMI) to try to put into the public domain pre-competitive data that pharma companies developed in the past. “It is a really interesting project that we are just kicking off, but I think it shows there is an appetite and willingness from many companies now,” Smith said. “If those data sets do not have any intrinsic commercial value to a company, yet there is value to other researchers to use them, then they should be placed in the public domain.”

Smith said ELIXIR was a place to store

data and that it was not a competitor for drug developers. “What often happens, especially in large companies, is that they struggle internally to make use of their data because of the large data volumes and the lack of interoperability between them,” he said. In the past, if there was not an immediate commercial value for data generated, then the data sets often just disappeared. “By opening these data and making certain aspects available, it could mean that someone else could come in and use them for answering scientific questions.” It is not a one-way street. Many large pharma companies regularly ingest into their own pipelines data from resources such as ChEMBL.

ChEMBL is a database of bioactive compounds that focuses on interactions between small molecules and their macromolecular targets, including medicinal chemistry, clinical development and therapeutics data.

ELIXIR is primarily helping industry to use public data, but at the same time, if industry does have data that can go into the public domain, then it can store those in ELIXIR’s repositories. Still, in some companies there are individuals who are less keen to make data open and the cultural change takes time. Lauer highlighted IMI and the FAIRplus project, particularly, as an example of how some pharma companies see the value in open science.

## What Is FAIRplus?

To gain maximum benefit from the growing amount of life sciences data available today the information needs to be available to researchers. However, it is often inaccessible, annotated inconsistently and difficult to share because it is in proprietary formats.

The FAIRplus project aims to develop tools and guidelines for making life sciences data “FAIR” – findable, accessible, interoperable and reusable. The project has 22 partners from academia and industry. It started in January 2019 and will run to June 2022. Lauer said, “The increased FAIRness of data will lead to a wider sharing of knowledge, greater opportunities for innovation, and more insights that benefit society.”

The FAIRplus consortium is supported by IMI, a public-private partnership be-

## WHY REUSABLE DATA MATTERS FOR FUTURE OF PHARMA

While studying epidemiology and medicine at the Vrije Universiteit in Amsterdam, Derk Arts realized there was no efficient application for simple and affordable data management. Founder and CEO of the business, he developed the first version of Castor's Electronic Data Capture (EDC) tool around 2011 as a cloud solution for capturing medical research data in clinical trials.

The EDC technology enables researchers to capture high-quality, standardized data and make it available for reuse. The technology platform has more than 30,000 users across 4,000 studies. The company, which has expanded to a team of 50 people, wants to make medical research faster and smarter by enabling researchers with user-friendly technology.

"Reusable data is a concept that exists in both the academic and commercial R&D settings," Arts told *In Vivo* in a recent interview. "Castor is trying to build a future where all medical research data can contribute to decision making."

Arts noted that "free text publishing" in scientific journals was currently the gold standard for data collection and the backbone of automated analysis. But he called this a "waste of data." He believes the future of medical research will rely on artificial intelligence (AI) to analyze data, and this will only be possible through better data collection and standardization. "Ultimately we want to be one of the key players in that brave new world of AI-led data analysis, where high quality research data can directly inform medical decision making."

Arts noted that in the commercial setting, having high quality reusable data was a competitive advantage. "AI is getting traction now in all fields, but its use is dependant on the quality of your data," Arts said, adding that Castor's technology saves time and limits the need for secondary data cleaning. "You can run artificial intelligence algorithms on data with our technology even when a clinical study is still running. Also, if data is useable as it is collected in a trial, it is easier to then automate parts of the trial. We really believe in the power of reusable data."

Castor's technology has mostly been used by academics, but Arts highlighted that pharma companies are seeking better solutions, especially those that are prioritizing the use of AI and machine learning technologies. He cited Johnson & Johnson as an example of a company that is expanding its use of tools in this space. In the academic sector, as an example, Castor's technology has been used in projects for rare disease registries.

There is more work to be done in the commercial space for the standardization of data. Arts said that companies were still focused on their top priority of getting a product to market but that interest and understanding about the importance of better data collection and storage was permeating through. "Reusable data might not directly impact an initial product approval, but it will help further down the road. It will take a few years until a company will truly benefit from making its data open and machine readable."

The terms "reusable" and "sharable" can still put off some companies, especially in the pharma industry where propriety data is instrumental to developing competitive products. But Arts was passionate when delivering his message about open data: "Open access doesn't mean direct access without approval," he said. "Very often our commercial customers think open research means making all their data public, which is not what it means. We must be very clear on what the boundaries are when talking about opening up data."

There is a demand in the academic sector for better collection tools for standardized and shareable data. Castor saw this gap in the market and used the academic sector as its incentive to develop its technology. Still, Arts said, "We will be ready to deploy this technology fully for commercial research when the sector is ready."

When speaking to potential commercial users, Arts said he focused on "the power of structuring data and information in a way in can be leverage five years from now for a different purpose." He said, "Companies need to make their own decisions on open data. I want to make sure we have the technology in place for when these companies decide to join the movement around data sharing and cooperation."

Arts noted that the commercial sector was already consuming open data to enrich its own internal data. But before the industry is really ready to share raw data, he said there would need to be greater incentives and guidance from governments and regulators. On a fundamental level, Arts said open sources and industry priorities would always clash. But there can be an ecosystem where the two co-exist, where the commercial sector can both consume and produce open data – if the benefits are significant enough.

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tween the European Union and the European pharmaceutical industry. Academic institutions work together with pharma partners to make their data FAIR. The focus is on pharmaceutically relevant data sets ranging from basic research to pre-clinical and clinically relevant data sets.

Pharma companies involved in the FAIRplus project include: AstraZeneca, Bayer, Boehringer-Ingelheim, GlaxoSmithKline, Janssen, Eli Lilly and Novartis.

### Learning From Other Sectors

In addition to large pharma companies using public data, and increasingly depositing their own data in the public domain, a new ecosystem of bioinformatics SMEs are emerging where their business model relies extensively on public data. Lauer highlighted Eagle Genomics as an example of a company actively using open data resources as part of its business model. The company is developing platform solutions for the health care, agritech, food and personal cosmetics industries. It uses open data resources offered by ELIXIR, including Ensembl and UniProt. “Eagle Genomics helps customers to find the right data sets and make use of them. The company helps companies mine data for whatever hypothesis they have,” she noted.

The interesting aspect of this company’s business model is that it can speed up research based on data that are already available. “Take a traditional pharma company, to test a hypothesis you first need to generate data, which means intensive lab work. To generate the data, you need biologists or chemists to generate those data sets and then you need to mine those data sets. There is a huge investment already at the start before you can test your research hypothesis,” Lauer said. She noted that bioinformatics companies bring a huge advantage to the R&D sector by being able to mine existing data sources.

Another company using open sources to its advantage is The Hyve in the Netherlands. It is an IT-type service providing open source bioinformatics or informatic solutions to scientists. The Hyve works with partners such as Dana-Farber Cancer Institute, and it is also involved in IMI projects.

These companies have been taking

public data and integrating them with data from their clients, which include large pharma companies. “But there are loads and loads of different sectors and different industry segments that rely on public data in different ways,” Smith said. “Take diagnostic companies, whether they are making sequencing technologies or proteomic machines, they have an interest in collaborating on joint standards for those data within the public domain.”

### Risks And Challenges With Open Sources

Smith said that within the high-risk life sciences industry, there was not, to his knowledge, a more “intrinsic risk” for small and medium life sciences companies in using open sources. Still there are challenges around integrating public domain data. “It is partially our role to help make sure that industry needs are considered by those developing new ELIXIR services. There are challenges in making data interoperable, but I don’t think that there is any higher – or we don’t see any higher – failure rates from these public bioinformatics companies than we do in other life sciences sectors. Some of them have grown very rapidly. He added that a lot of new machine learning and AI companies are heavily reliant on being able to compare against the public data sets.

One of the main challenges for using data efficiently and effectively is finding skilled bioinformaticians. “In the last few years there has been such a high demand for qualified bioinformaticians,” Smith said. He added that training courses provided through ELIXIR was one way of trying to address that issue. “A lot of the time the large IT companies are proactively recruiting well-trained bioinformaticians, so it can be harder for the small and medium enterprises to find well-trained staff.”

Altering traditional mind-sets within companies is a lingering challenge. Big data are not new but the technology to use, mine and analyze data has changed, particularly, how you can put data in relation with other data sets. “This is something that has grown over the years and many decision-makers in more established companies were not necessarily trained for this,” Lauer said.

The biggest bottleneck preventing the best use of open data in the life sciences sector is the lack of universal standards. “Agreeing on standards is critical so people can actually share data, put data in context or link different data types to each other,” Lauer said. “This is a matter of the sector coming together, building those standards, to solve the interoperability problem.”

When it comes to integrating research data with clinical data, there is still a lot of work to be done. “Pharma is still thinking through how this could be done. For example, all the electronic health records that are stored locally or often in different languages across Europe, how can these be mined in a way that integrates them with other data? It is a whole new area that everyone is really coming to grips with. It is not only an industry-specific challenge but something that both industry and the public domain are trying to work through right now,” Smith noted.

“The future is bright,” Smith said. “There are so many different opportunities. There is still a huge need to collaborate and coordinate to make the data interoperable. There are certainly challenges out there and that’s what we will be focusing on. We are working with industry to make sure we understand its needs so that it can feedback its requirements to the partners in ELIXIR.” Smith expects to see many more bioinformatics companies being created, merging with other companies and being bought out by larger companies.

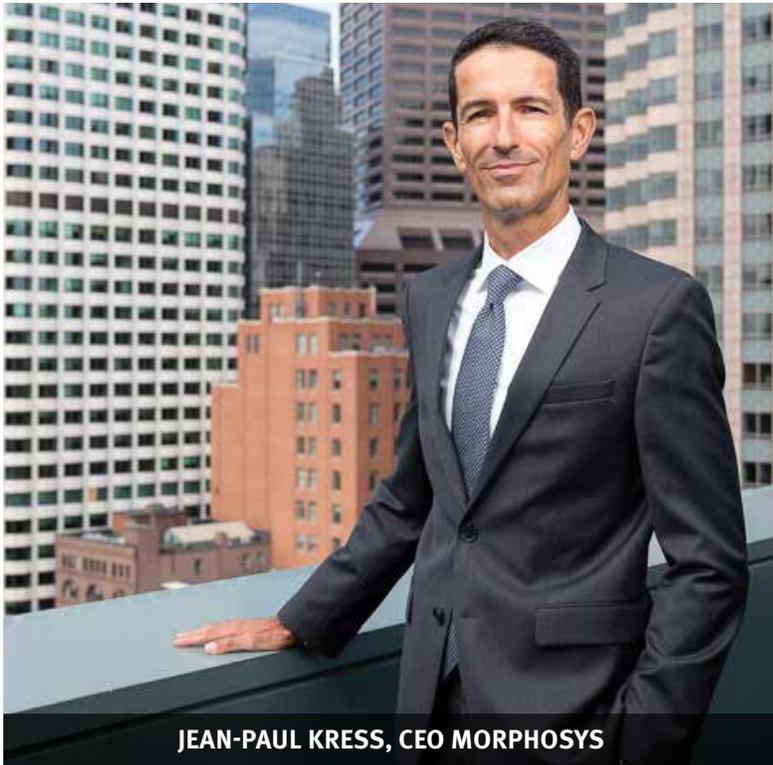
Lauer added that technology was improving and it was becoming cheaper to generate data. “Looking forward, there are a lot of national genomics initiatives in the health care sector generating data at scale which will help in areas such as rare disease where traditional cohorts have been smaller in nature. We will definitely see improvement in scientific discovery, and importantly benefits for society across the life sciences.”

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# MorphoSys: A European Champion Crossing The Biopharma Rubicon



JEAN-PAUL KRESS, CEO MORPHOSYS

The Munich-based company is one of a handful of mature European biotechs poised to enter the big league. New CEO Jean-Paul Kress talks to *In Vivo* about the firm's next generation antibody platform and why US expansion is crucial to its future.

## BY ANDREW MCCONAGHIE

There are virtually no examples of European biotech companies breaking into the “big league” and becoming fully fledged biopharma companies, unlike the many US success stories such as Amgen, Alexion and Vertex.

But that looks set to change, with a crop of hugely promising companies, including the likes of Denmark's Genmab AS, Belgium's Galapagos NV and Germany's MorphoSys AG, all reaching maturity in 2019.

While many European companies have reached this stage before, only to be gobbled up in big pharma M&A, it looks like these CEOs and boards may resist any M&A overtures and keep their eyes set on long-term growth and expansion.

**M**orphoSys, based in Martinsreid near Munich, has been around for a full 27 years, and was until this year led by its co-founder Simon Moroney. He oversaw the patient and painstaking development of the company's R&D expertise over those decades. The strategy first focused on providing its next-generation antibody technology to big pharma, but MorphoSys now possesses an impressive pipeline of partnered and in-house molecules.

The company has a potential blockbuster on its hands, in the shape of its lead in-house candidate tafasitamab (formerly MOR208), a novel treatment for diffuse large B cell lymphoma (DLBCL). It was granted Breakthrough Therapy Designation by the US FDA in 2017, and after generating compelling data in Phase II trials this year, is poised for filing in the US by the end of 2019.

Following a successful IPO on Nasdaq in April 2018, which raised \$208m, the company is also well financed. MorphoSys is building up commercial operations in the US and Europe in anticipation of tafasitamab's US approval by mid-2020.

All of this has made the company “one to watch” for biopharma analysts. They have also endorsed its selection of Jean-Paul Kress as the new CEO. A French national who shares MorphoSys' European culture, Kress also has lots of experience in commercializing major products in the all-important US market. But of course, there are still many potential pitfalls for a company in this position.

The first of these is an unexpected objection or rejection from the FDA, which would set it back against its competitors in the fast-moving hematology-oncology field. Or after approval, a disappointing commercial launch for tafasitamab – something that is always a possibility when expectations are already so high.

Talking to *In Vivo* just weeks after taking the helm at the company, Kress said he was dividing his time equally between Germany and the US as he tackled key challenges. He said three things topped his list:

- ensuring a “flawless” filing of tafasitamab with the FDA before the end of 2019;
- establishing a strong commercial base in the US; and
- sealing a partnership to maximise the drug’s revenues, which he described as a “pipeline in a product.”

Asked about what kind of alliance MorphoSys was looking for, and whether it might include the prized US market, Kress did not want to be pinned down. “I have no religion on this ... my only goal is to maximize the value of the product,” he said. “We’re in a very strong position, there is a lot of interest from what we call the ‘strategics’ and potential partners. We’ll do what makes sense for the patients and for our shareholders.”

Nevertheless, MorphoSys is likely to retain a share of rights in the US, as it has already established a US headquarters in Boston, and has assembled a medical science liaison (MSL) team ahead of an anticipated mid-2020 FDA approval. The company is also laying the groundwork in Europe, where it expects to file around the same mid-year period.

Its need to find a big pharma partner with expertise in oncology has led analysts at Brian Garnier & Co. to suggest that **AstraZeneca PLC** would be a good ex-US partner, based on its presence in hemato-oncology with Calquence (acalabrutinib).

### Next Generation Antibodies

The next-generation antibody has been dubbed by some a possible “CAR-T killer” in the diffuse large B-cell lymphoma (DLBCL) space, after posting stellar results in a Phase II trial, with analysts forecasting peak sales in excess of \$1bn. Results from tafasitamab’s Phase II L-MIND study showed it has great promise in relapsed and refractory (r/r) DLBCL patients who are not eligible for high-dose chemotherapy and autologous stem cell transplantation. Released in May, these data showed that in combination with Celgene’s Revlimid (lenalidomide), the drug hit its primary endpoint, producing an overall response rate of 60%, with 43%

achieving complete responses, indicating the disease had disappeared entirely.

The results in the 80 patients were impressive, producing similar efficacy results to those produced by the recently approved chimeric antigen receptor T-cell (CAR-T) therapies, Novartis’ Kymriah (tisagenlecleucel) and Gilead’s Yescarta (axicabtagene ciloleucel), but with fewer serious side effects. Tafasitamab’s also benefits from far less complicated – and costly – drug administration.

Tafasitamab is an antibody targeting CD19 (the same target as these CAR-T treatments), with an engineered enhancement of its “constant Fc” region boosting the body’s immune response compared with traditional mAbs. This Fc-enhancement has been shown to lead to a substantial potentiation of antibody-dependent cell-mediated cytotoxicity (ADCC) and antibody-dependent cellular phagocytosis (ADCP; *see Exhibit 1*).

Piper Jaffray & Co are among analysts predicting big things for MorphoSys and forecast total revenues of €715m (\$783m) by 2024 and €1.27bn (\$1.39bn) by 2028. This would be based on tafasitamab in DLBCL and further B cell malignancies such as chronic lymphocytic leukemia (CLL) and small lymphocytic lymphoma (SLL).

### Polivy Already Launched

But tafasitamab is not the only new antibody entrant to the market. **Roche’s**

Polivy (polatuzumab) gained US approval in June, in the same relapse and remitting DLBCL patient segment. Roche’s drug is a first-in-class monoclonal antibody, which achieved a similar 40% complete response rate in patients.

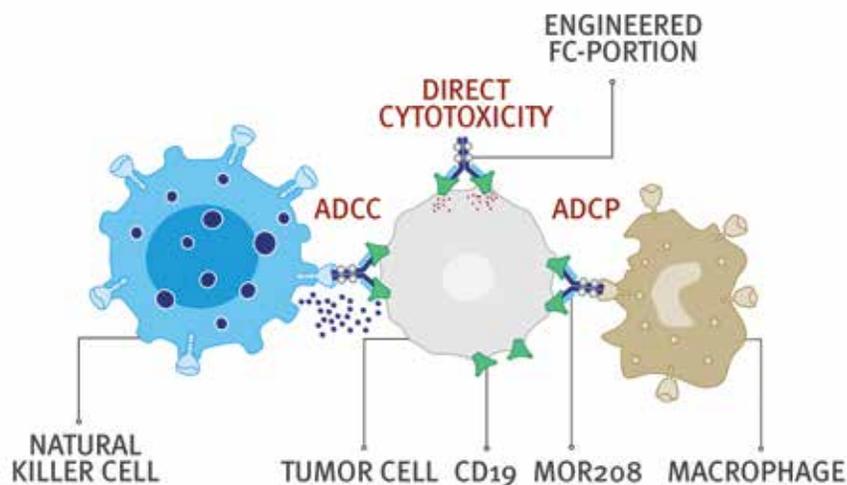
A licensing deal would help the company maximize tafasitamab’s potential in this highly competitive market. The risk is clear: even with experience management on board, new biopharma companies launching without marketing partners often end up with underwhelming commercial results, such as Clovis Oncology with Rubraca (rucaparib) and Intercept with Oclavia (obeticholic acid).

The company has a couple of important readouts before the end of 2019, starting with an interim look at the B-MIND study, expected in November. This Phase II/III study is evaluating tafasitamab in combination with bendamustine versus rituximab and bendamustine. A futility analysis is likely to result in the study continuing in all-comers or biomarker selected patients. And the study could help bolster MorphoSys’ position in talks with potential commercial partners.

Meanwhile a synthetic control arm of L-MIND will be submitted to be a latebreaker abstract at the American Society of Hematology (ASH) congress in early December, with the drug filing with the FDA expected shortly afterwards. The company also has the

### Exhibit 1

#### Enhanced Immune Response Thanks To An Engineered FC-Portion



SOURCE: MorphoSys

molecule in studies in relapsed or refractory CLL and SLL.

**A Pipeline Full Of Potential**

One of the most remarkable things about MorphoSys, when considering a biotech of its size, is the breadth and depth of its pipeline. It has more than 100 distinct molecules in its pipeline, giving it what it calls “one of the broadest pipelines in the biotechnology industry.” This includes candidates for cancer, Alzheimer’s disease, infectious diseases, cardiovascular dysfunction and inflammation.

This means MorphoSys is in an enviable position, giving it ample opportunities across a range of areas of unmet need. At the same time, it also runs the risk of stretching itself too thinly in order to fund this very broad pipeline. It is a danger that Kress has acknowledged: “No doubt that there will be a need to keep a close eye on that, and continue to be very discriminating on capital allocation.” Meanwhile, the company has also benefitted from partnerships in R&D.

In fact, MorphoSys already has a marketed product on its hands from this route: it discovered Janssen’s Tremfya (guselkumab) for moderate to severe plaque psoriasis, which gained FDA approval in July 2017. The drug is tipped to become a blockbuster, and is already generating a steady income stream for MorphoSys, which it will be able to rely on as it invests in R&D and moves towards profitability.

Among the many other pipeline assets is MOR106, an IL-17C inhibitor for the treatment of inflammatory diseases, that is currently in clinical development for atopic dermatitis. MorphoSys jointly discovered and developed the antibody with Galapagos before signing an exclusive license agreement with Novartis in July 2018, with the Swiss pharma company taking on all responsibility for R&D, manufacturing and commercialisation costs.

**US Biotechs Still Have An Advantage**

Kress has plenty of experience in deal-making and major launches; knowledge which will be vital for this crucial period at MorphoSys. Prior to joining MorphoSys, Kress served as CEO of Syntimmune,

*MorphoSys is “in a very strong position, there is a lot of interest from what we call the ‘strategics’ and potential partners.”*

**JEAN-PAUL KRESS**

a clinical-stage biotechnology company developing autoimmune disease therapies, which was acquired by Alexion in November 2018 in a deal worth up to \$1.2bn.

Before that he was Sanofi Genzyme’s head of North America operations, where he played a key role in launching Dupixent (dupilumab), a biologic atopic dermatitis treatment that has become one of the biggest launches of recent years.

This experience also gives him valuable insight into why Europe has so far been unable to match the prodigious biotech company production line seen in the US. So, why does the US still lead Europe? “Knowing the environment on both sides of the of the pond, I would say the difference comes down to financing and the knowledge of the investors,” said Kress. “In the US you have very specialized, educated and knowledgeable investors” focused on life sciences.

“We don’t always have that in Europe. So, there is a bit of a knowledge issue here that we [in Europe] have to address,” he said. “The US market tends to reward innovation, and that capital gets returned to investors, who tend to reinvest in the sector. It’s a virtuous circle which works really well. But it is a mentality as much as anything.”

**Antibody Technology**

Nevertheless, Kress said European science was easily a match for the US, and he added that he was impressed by the quality of the development work and the people in MorphoSys’ labs in Germany. The company continues to

develop proprietary drug discovery and development platforms that could help it differentiate its “next-generation” antibodies from those from the many competitors in the field.

One key technique is dubbed ENFORCER, which stands for enhanced format for cancer eradication. This refers to the structural modification of the antibodies that brings about better recruitment of effector cells and increased elimination of cancer cells.

Behind this is MorphoSys’s proprietary HuCAL (Human Combinatorial Antibody Library), a recombinant antibody technology that enables the generation of therapeutic and diagnostic antibodies – including those binding to difficult antigens.

MorphoSys also has Ylanthia, which it claims is the industry’s largest antibody antigen-binding fragment (Fab) library to date. It said the library’s unrivaled structural range and diversity allowed for a greater number of possible bindings for promising target molecules, and thereby the ability to identify more opportunities.

Kress said this antibody expertise was “best in class, and sometimes you don’t find that in the US.” He added that MorphoSys’ R&D operations in Martinsreid was attracting talented scientists from China and beyond, demonstrating its global reach. MorphoSys has earned the confidence of investors because of its strong science and discipline management, which puts it on a level playing field with any US-based biotechs.

But for European-based start-ups, there is unquestionably less funding. “When you haven’t crossed the Rubicon, yes, it’s a bit more difficult here,” Kress said. “But when you have gone through this challenge, and you’re well capitalized ... then you can leverage the best of both worlds.”

There is still a long way to go before MorphoSys, or any of its similarly well-placed contemporaries, can aspire to match Amgen or Genentech. But there is no question that European biotech has reached a new level of maturity, which could lift confidence and investment in the sector in the next five to 10 years. ❦

**IV124350**

**Comments:**  
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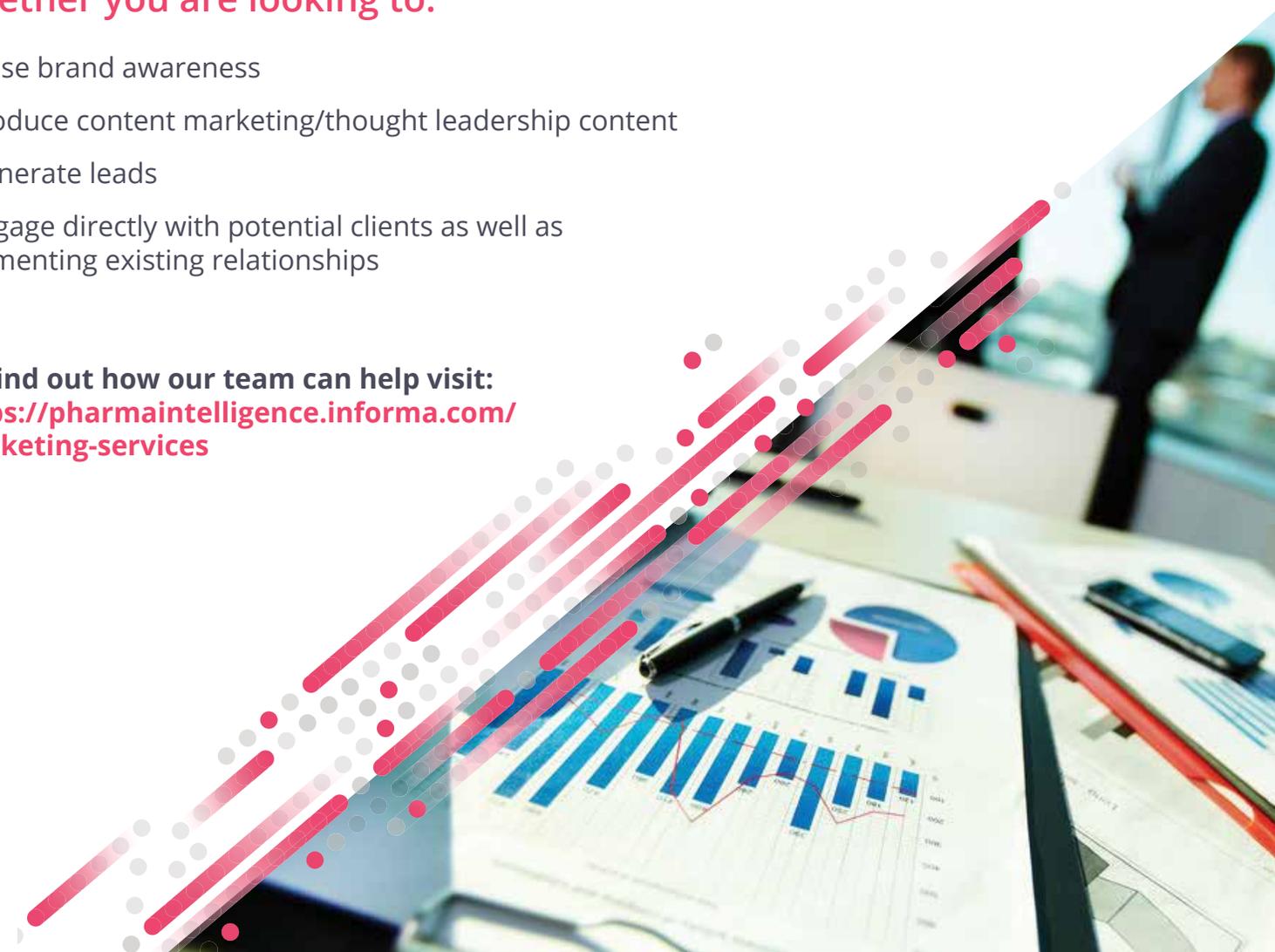


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# PureTech's Problem-Solving Skills Target The BIG Picture



PureTech Health has amassed niche expertise and built a unique structure that allows it to develop technologies for unmet needs, in the separate but related areas of brain-immune-gut. And now, with the experienced ex-Sanofi CEO Chris Viehbacher as interim chair, PureTech can target a smooth path to its next growth stages in the “BIG Axis.”

## BY ASHLEY YEO

PureTech Health PLC is exploiting a strategy of developing and commercializing highly differentiated medicines for unmet needs through in-house and affiliate divisions, whose remit extends to therapies besides its biopharma strengths in the BIG Axis.

This model has allowed the company to create a broad and deep pipeline of 24 product candidates, being advanced both internally and through its affiliates.

With former Sanofi CEO Chris Viehbacher elevated from board member to interim chair, PureTech has a guaranteed steady hand on the tiller. The appointment followed swift action by PureTech to distance itself from any associations with board member Joichi Ito, after the US press recently revealed his inappropriate financial ties with a public-eye figure facing criminal charges.

PureTech is creating a business out of taking a problem-based approach to unmet needs in health care. Its record to date reveals a company that has a skill in being quick and effective at deciding on the right pathway, and selecting programs and technologies that might, to some, seem unexpected.

These problem-solving skills have been called into play for distinctly different reasons recently, after it was reported that MIT Media Labs head Joichi Ito had been maintaining financial ties between the Labs, and the financier and convicted sex offender Jeffery Epstein, whose long-running legal troubles worsened in July 2019. It appears that for PureTech, the precise problem was that Ito was also a board member of the Boston, US headquartered group.

But PureTech founder and CEO Daphne Zohar acted decisively. After Ito resigned his MIT post, she moved to end PureTech's relationship with the former executive. “Given circumstances ... we agreed that [Ito's] resignation from PureTech was appropriate,” she said in a statement. It was swift and final.

In a related move, she elevated seasoned pharma industry strategist and board member Chris Viehbacher to interim chair, a role where he could accentuate the scientific work of the group and put the focus squarely back onto PureTech and its programs.

PureTech explained these programs to *In Vivo* during a recent Jefferies Healthcare conference in London, where Eric Elenko, PhD, chief innovation officer, and Joep Muijers, PhD, chief financial officer, explained what made PureTech different. They also detailed how the company had honed its portfolio target into three separate – but linked – therapy areas, and how it had established the underlying programs and platforms for a pipeline of 24 product candidates (of which 14 are at the clinical stage). One of them is FDA-cleared: Gelesis's Gelesis 100/Plenity has claimed to be the only

prescription weight management product cleared for use in overweight adults with a BMI as low as 25kg/m<sup>2</sup>, with or without comorbidities (such as hypertension, type 2 diabetes, or dyslipidemia).

The underlying program and platforms that resulted in this pipeline of product candidates were initially identified, or discovered and then advanced, by PureTech's team through key validation points based on unique insights into the biology of the brain, immune and gut – BIG – systems, and the interface between those systems. This is referred to by PureTech as the BIG Axis, where the lymphatic system is key.

PureTech also maintains a busy program of outreach and involvement with third parties, the latest of which, in October 2019, was the acquisition of the remaining 10% in Ariya Therapeutics, Inc., from the co-inventors of its platforms and associated universities and advisors. Now renamed PureTech LYT, the subsidiary has four technology platforms in immuno-oncology, synthetic lymphatic targeting chemistry, milk exosomes and meningeal lymphatics.

PureTech also has a third strand: de-risking the non-core applications of internal discovery platforms at major partner companies, such as Boehringer Ingelheim International GmbH and Roche, via non-dilutive funding sources, including partnerships and grants. The major names that PureTech partners with also include Shionogi & Co. Ltd. and Purdue Pharma LP.

## Two Divisions

Elenko, among the first employees at PureTech, has led the development of several programs at the company, including those within the “Founded Entities” division, Akili Interactive Labs Inc., Gelesis Inc., Karuna Therapeutics Inc. and Sonde Health. The full list of Founded Entities extends to Alivio, Follica, Vedanta Bioscience, Vor, resTORbio and Entrega. These affiliates are mainly in the Boston area, while Akili Interactive Labs is split between Boston and the San Francisco Bay Area; and Gelesis has manufacturing and R&D in Italy.

Elenko's role includes helping to run start-up programs for the Boston, US-based, UK-public listed company. The



ERIC ELENKO



JOEP MUIJERS

company, founded by Zohar, started in biopharma and branched out into the device area. “The idea behind PureTech and its basic approach is to be problem-solving: we start with a problem that represents a great unmet need in health care,” Elenko explained.

“We'll convene a group of leading experts and brainstorm about the best approach to solving the problem.” That becomes the basis to proactively look for solutions that typically originate in academia or industry. PureTech has done two spinouts on the biopharma side, having taken three compounds, including those from the Founded Entities.

The company is more dominant in biopharma than medtech, with a focus on the CNS, immune system and GI trio – brain, immune, gut – including the interaction between those systems. A study of the microbiome shows a clear link between the microbes in gut and in the immune system. PureTech's Vedanta Biosciences affiliate has been studying that link, having pioneered the idea of the interaction.

## Unmet Needs

Affiliates that are pursuing solutions that are seen as devices – at least from a regulatory viewpoint – also center their projects around unmet need. For

instance, in the case of Gelesis – an oral hydrogel therapy that induces feelings of fullness without adding calories – there was a recognition of an unmet need in the treatment of obesity and co-morbidities. This represents a tremendous public health problem that is only increasing.

In the case of Akili, PureTech was interested in the unmet need as regards cognition. In ADHD, one of the central issues is attention. Akili seeks to bring together neuroscience with the latest technology and video game entertainment to challenge the status quo of medicine. It is essentially a digital medicine “delivered through a proprietary and captivating video game experience.”

With Sonde's technology, PureTech was pursuing the idea of detecting disease and being able to inform the patient (and other related stakeholders) in a “low- to no-burden” way. “That would have a tremendous impact on patient care, and from an economic viewpoint.” The ability to intervene earlier would obviously prevent more catastrophic effects down the line.

These “device” projects were described by Elenko as primary interventions for the illnesses they target, not merely additions to the PureTech portfolio.

“One of the great ideas with chronic illness is intervening at a point where you can make an impact prior to a very serious sequela that's difficult for the patient and more costly for the system,” said Elenko. Those sorts of intervention are of becoming more common; as seen, for instance in the UK NHS's Long Term Plan, issued in January 2019. From a medical viewpoint, the value of prevention is well understood, but the driving forces that are making it increasingly a reality are notions of economic reality and cost-effectiveness.

## Product Focus

The product mix that PureTech is advancing is in some way a unique blend for a company numbering approximately 55 or so staff (direct PureTech employees, i.e. excluding those at affiliates). “The way we look at products selection goes back to the unmet need argument: if you have a technology looking for a problems to solve, that *can* work, but it's often better to look at the problem and seek the ideal solution,” said Elenko”

## WHO'S WHO

Prior to joining PureTech **Eric Elenko** was a consultant with McKinsey and Co., where he advised senior executives of Fortune 500 and specialty pharmaceutical companies on licensing, mergers and acquisitions, R&D strategy and marketing. His BA in biology is from Swarthmore College, and his PhD in Biomedical Sciences from University of California, San Diego.

**Joep Muijers** was responsible for investing in publicly traded companies. Notable investment companies that were acquired by large pharma include Ablynx, Colucid, InterMune, Kite Pharma and NeuroDerm. Others have become leaders in their respective areas, such as Evotec, Genmab, GW Pharmaceuticals, MorphoSys and Neurocrine. He was previously director of corporate finance and capital markets at Fortis Bank, now part of ABN AMRO. His PhD in molecular biology is from the European Molecular Biology Laboratory (EMBL) in Heidelberg, Germany. His Master's Degree in Biochemistry is from the University of Nijmegen, the Netherlands.

**Chris Viehbacher** is managing partner of Gurnet Point Capital. Prior to joining Sanofi, he spent 20 years at GlaxoSmithKline, ultimately serving as president of GSK's North American pharmaceutical division and as a member of the board of directors of GSK PLC. He began his career with PriceWaterhouseCoopers LLP and qualified as a chartered accountant in Canada. He has chaired the board of the US Pharmaceutical Research and Manufacturers of America (PhRMA) and been president of the European Federation of Pharmaceutical Industries and Associations (EFPIA).

Two products have been submitted to the FDA, the cleared Gelesis 100 (also called Plenity) treatment, and one from Akili (AKL-To1) for treatment of pediatric ADHD. Akili's early academic work came out the University of California San Francisco. PureTech's advanced affiliates have matured to the point where they have their own management teams and officially file their own applications with the FDA.

The company has two divisions: affiliates, which are separate legal entities; and internal programs driven by PureTech itself. Over time, ownership of the affiliate might get outside capitalization from other groups. Are present, Akili is 34.9% owned by PureTech; Gelesis, 19.5%; and Sonde, over 55.9%. Sonde completed a \$16m series A financing round in April 2019, led by M Ventures (the corporate venture capital arm of Merck KGaA).

"When we start something, it's ours, and it evolves over time." PureTech has the option of taking these companies public at some stage, for instance, resTORbio was listed as is a public company on Nasdaq in 2018. Equally, there could be a trade sale of a company to maximize value.

Shared resources, institutional knowledge, cross fertilization and best practice ideas are all benefits to the company affiliate of the structure that PureTech oversees. Sharing knowledge about the BIG Axis therapeutic domain helps build scientific networks. "That synergy is extremely helpful," said Elenko.

The already-cleared Gelesis 100 is regulated as a device by the FDA, and is now being taken along the CE-marking process. The company is aware of the EU Medical Device Regulations (MDR)-induced pressure on notified bodies' file processing capacity. This is leading to both increased cost and uncertainty, given the lack of auditing bodies available to process product submissions under new regulation, which come into force on 26 May 2020.

### Akili's Digital Medicine

Akili leads the way in digital medicine and solving cognition problems. Depression, cognitive issues and MS, are potential applications, after ADHD. PureTech sees this as a huge unmet need where

there are not many pharmacological options. "We were interested in the idea of having something that is extremely safe, and non-invasive, particularly given the wide population this might be applicable to," Said Elenko.

The basic science behind Akili is the idea of improving cognitive interference, which is processing of multiple streams of information at the same time, and processing out "noise." This device essentially does that as a therapeutic intervention, whereby the user performs two different tasks that are overlaid in a very specific way. They increase in difficulty for the user, and includes complex psychometric staircasing algorithms that self-adjust, offering "training."

"Akili is a very serious therapeutic," Elenko stressed, that underwent a pivotal trial with a gold standard endpoint of the TOVA (Test of Variables of Attention) measure of attention. "This is a medical product who goal is to be integrated into mainstream medical practice."

Akili's therapeutic video game technology is regulated as a medical device by the FDA, and has value in addressing an unmet need, so can be viewed in the context of being reimbursed. The big questions reimbursement authorities ask are: Is it a real product? And does it work?

Digital product reimbursement is still in its early phases. But Elenko acknowledged that lately there has been a drive by the FDA to be more at the front of digital technology. "It's a nascent field, but also a positive environment. And these are mainstream medical products we are developing," Elenko asserted.

### The Great Potential Of Voice

Sonde's technology is at an earlier point in its commercial development, but like the others, is also a product for use in mainstream medicine. It is based on the premise that the voice can be a physiological marker of dysfunction in the body. "We're talking about acoustic features – how does someone sound, not the actual words they're saying, said Elenko." Sonde takes in vocal data, correlates it with gold standard measures of a disease – e.g. depression – and then carries out machine learning on the variables, which provide models to be used in various contexts.

“We identified the great potential of voice, talked with leaders in the field all around the world and identified technology that came from MIT Lincoln Laboratories, which we licensed in.” Sonde filed its own IP and did its own development, which is a typical arrangement within PureTech. “The starting point is that we license in IP, then build on it,” said Elenko.

The next stages for Sonde include pursuing regulated and non-regulated health claims. “For example, we would be interested in screening for depression, which is a regulated claim. We see ourselves going down that path,” said Elenko, stressing the cutting-edge nature of the technology which works on devices already owned by the individual patient – meaning no need to purchase new hardware or use wearables.

### A Wide Portfolio Within BIG

PureTech keeps its focus on the area of BIG when deciding where and how to start a new project. It sees itself as a biopharma company, and when products fall under the “device” category, the company views them as new sorts of therapeutics. Sonde, Gelesis and Akili fit into this category of “novel,” in being new types of therapeutics. And in starting with the problem-based approach, PureTech and affiliates have options to move forward via either the internal division, which has more of a focus on immune, and in particular, a high concentration of work related to the lymphatic system; or via the affiliates division, which also has activity around the brain and gut, as well as immune.

The PureTech board is a key tool in profiling and deciding on new areas to move into. It is a combination of top entrepreneurs and academics, such as Bob Langer, ScD, who has started up numerous companies; Bob Horowitz, PhD, who is Nobel Laureate in Medicine; and Raju Kucherlapati, hD, who was a co-founder of Millennium Pharmaceuticals (acquired by Takeda for \$8.8bn) and Abgenix (acquired by Amgen for \$2.2bn). Industry experts include the new interim chair Chris Viehbacher; John LaMattina, PhD, who once headed up R&D at Pfizer; and Ben Shapiro, who led external R&D at Merck & Co. Inc.

“

*“By taking a proactive approach to a problem and a blank sheet of paper, we can identify things that are completely novel before the rest of the world knows about them”*

– Eric Elenko

”

Besides the board, PureTech has a large network of scientific advisors, and its own internal team. “We are constantly thinking about unmet needs and what new area of technology are on the cutting edge,” said Elenko. For example, PureTech started working with Adam Gazzaley, co-founder and chief science advisor of Akili Interactive Labs, prior to the publication in 2013 of the technology (then called NeuroRacer) in *Nature*, as a cover story. “There are a number of similar initiatives where, by taking a proactive approach to a problem and a blank sheet of paper, we can identify things that are completely novel before the rest of the world knows about them,” said Elenko.

### Unique Business Model

It is an approach that gives the company the flexibility to reflect on whether the solution to a health problem might not be a small molecule, but rather a regulated medical device. “It’s a very unique model,” said PureTech CFO Joep Muijers, adding “I don’t think any other company has such a dual structure.” There is a nu-

ance, however. The internal pipeline that PureTech has developed means it can still source technologies via the best technologies for lymphatics and the immune cell trafficking, for instance. “We’re still building around that, but we’re very well capitalized,” said Muijers. That allows PureTech to hold onto its assets much longer than it has in the past.

“Our model had always been: identify technologies, set up entities, and grow them. And when the capital needs became too large, we would go out for external financing,” said Muijers. But with \$149.2m on the balance sheet at the end of the first half of 2019 (PureTech only), he said, “This is enough to do anything we want to do through to the first quarter of 2022.” On top of that, in H2 2018, PureTech struck a \$36m R&D deal with Roche. This amount includes upfront payments, research support and early preclinical milestones. PureTech Health will be eligible to potentially receive development milestone payments of over \$1bn and additional sales milestones and royalties for an undisclosed number of products.

And on top of that, if it is the right time, right price, PureTech has the option of monetizing any stake in its affiliates. Muijers added that PureTech had the optionality to do that. “We do expect that in time, we’ll be transitioning a little more towards the internal pipeline.”

### Next Stages For PureTech

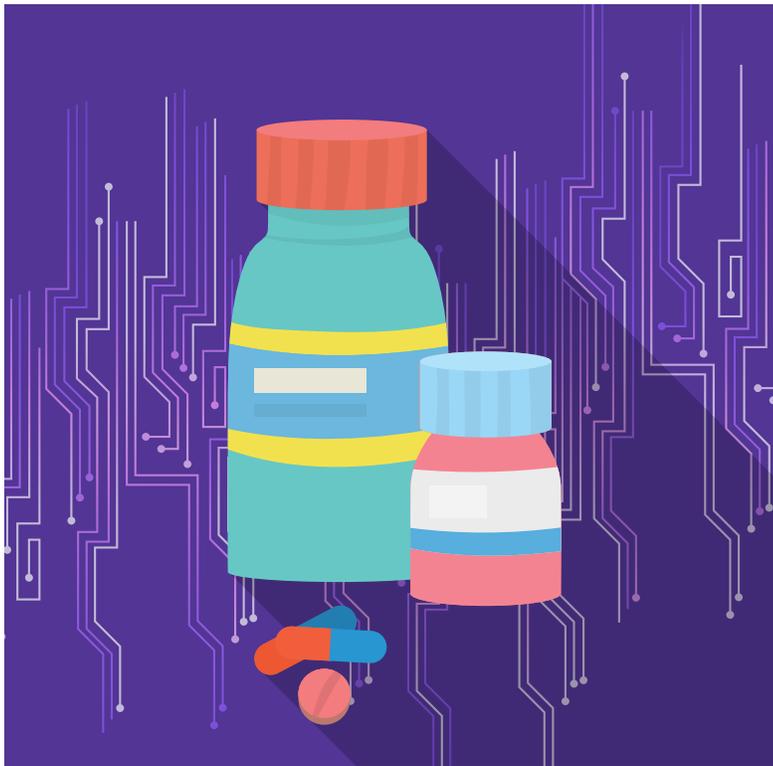
PureTech will continue to leverage ideas from the affiliates, ranging from preclinical right up to in-front-of-the-FDA products. Within PureTech, a harder drive forward is developing in lymphatics, which is seen as an area of great possibility and opportunity, and where PureTech said it “would expect to be spending additional time.” It will also continue to pursue partnerships with large companies, along the lines of the agreements done with Roche, with Johnson & Johnson, via Vedanta Bioscience, and others listed above. “That will depend on what makes the most sense in terms of maximizing value,” said Elenko. ❖

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#### Comments:

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# Digital Labels: Reimagining Product Information In The Digital Age To Improve Patient Outcomes



The rules behind product labeling have not undergone any significant revisions in the major markets of the US and Europe for many years, but is this about to change? In the last decade two seminal shifts have occurred which suggest this may be the case.

**BY AMY CHEUNG, OLIVER STECK, MUKESH SINGHAL, SHIMON YOSHIDA AND SHARON GORMAN**

The label on the outer packaging and leaflet provided within the box is the primary, and sometimes only, message that patients and practitioners see from the Marketing Authorisation Holder (MAH) relating to the safe and effective use of the product.

While the content and format of the product information can vary according to local or regional regulations, it is always subject to a rigorous assessment by Health Authorities (HA).

The final text of a label or leaflet is no small effort—it can span a professional lifetime of data collection, aggregation and interpretation.

**T**he rules behind labeling have not undergone any significant revisions in the major markets of the US and European Union (EU) for many years, but is this about to change? In the last decade, two seminal shifts have occurred which suggest this may be the case, and there are early signs from other regions that the labeling landscape may be changing, for example as in Japan.

The first shift is driven by the acceleration of digital disruption – life sciences is no exception. Traditional players, including pharma and medical device companies, are now joined by a large number of technology providers, digital start-ups and other entrants. Indeed, even familiar consumer technology companies like Apple, Google, and Amazon are now at the forefront of health care disruption.

The second shift is around patient-centricity, where commercial success is increasingly determined by the “customer experience.” In the age of consumerism, digital products designed for busy lifestyles that are accessible through intuitive, personalized, user interfaces which are trusted, reliable and easy to use are frequent winners in crowded markets.

*In Vivo* examines whether labels are next in line for digital transformation, with an increased emphasis on the consumer. There are inherent challenges to conveying product information, and risks that regulators must consider when changing the rules, such as accessibility, low digital literacy, patient confidentiality and other legal concerns. Looking beyond challenges, there are potential benefits to patient health and correct product use that can be realized through a digital transformation of labeling.

## Current Challenges

It is well-established that medication errors are directly correlated to significant morbidity, mortality and costs that are detrimental to patient care. A study recently conducted by the Institute of Medicine concludes that poor labeling and the use of certain terminology is a central cause for medication errors and a rise in adverse events and poor health outcomes (Jeetu, 2010).

Labels provide guidance and act as a reference point for patients and health care professionals to safely adhere to proper regimens when taking or administering a medication. It is naturally challenging to do this well, but current rules and industry practices in most countries do not generally encourage significant patient input in the process of determining the composition of this crucial information, particularly following Health Authority approval when patient experience with the product is rapidly rising. In addition to critical information on dosage and usage, labels also contain detailed information on safety precautions, as well as additional background information, e.g. on clinical studies – much of which may not be applicable to an individual patient. MAHs typically do not provide individualized context for different patient populations, unless such situations have been specifically studied. While there are readability guidelines for labels in the EU, guidances around the accessibility, presentation and design of labeling information as well as user testing, these do not prevent the label from being multiple pages with small font, particularly where multiple languages are required.

A non-specific and lengthy label means that, in order to get understandable information on their medicines, patients are more likely to reach out to their health care professional (HCP), or the pharmaceutical companies' medical information contact number to ask questions, or else try and conduct research themselves, often using online resources. Reliable product research – finding, navigating and understanding product information – can be difficult for patients, especially for those with lower health literacy. Information available on the internet is often not written by the MAH and can be

out of date or inaccurate. These barriers combine and can contribute to challenges for patients in adhering to their regimens and taking their medications properly.

In addition to being complex, information contained with the label is also dynamic and may require frequent updates. When a pharmaceutical company collects new information on a product, such as patient safety data, subsequent updates to the product label are often required. Such updates may inform prescribing decisions, describe new side effects, or make regimen changes. The process to incorporate these updates into product labels and packaging, however, is lengthy and time consuming – moving through the necessary steps to update the labeling information, receive HA approval, generate artwork revisions, print and package mean that it can take months before updated information is available in or on the product packaging.

Ultimately, the issue in the current labeling paradigm is that information within labels is not well conveyed to patients. While some of this is due to the inherently complex and dynamic nature of labels, improvements are possible if labeling could be written and disseminated in a way that puts the needs of patients first rather than to meet a legislative prerogative. Aside from patients themselves, it is industry that has the greatest stake in communicating their product information in the way it will be best-received. If patient needs are at the forefront in considering how best to communicate this crucial information, pharmaceutical companies will gain a powerful tool to further their mission of improving the health of their end-users.

## The Opportunity In Digital Labels

Applying the concept of patient-centricity to labels would represent a fundamental shift away from labels focused more on providing mandated drug product information, to providing meaningful and targeted product insight to benefit all patients. The ultimate goals of this shift are for patients to become better-informed about the medication and treatments they are receiving, and to take greater ownership of their own health care, and ultimately their own health outcomes. Emerging digital technologies

have had success in other industries, especially where a complex process has been simplified by the use of technology, and these technologies represent a key opportunity to break the one-way paper-driven paradigm in the pharmaceutical industry as well.

## Value In Patient-centricity

Emerging digital technologies now allow for digital labels to dynamically change, showing the latest, HA-approved information in the most patient-specific and user-friendly way, thus enabling patients to better manage their own health. For example, by incorporating patient health data such as personal health records, digital labels could ultimately provide more applicable and relevant information to the patient.

To address the verbosity and length of drug labels, a digital label could be tailored to only show the indications directly related to a patient's diagnosis, removing the extraneous additional indications associated with their medication, and possibly even replacing them with important notes from a recent doctor's visit. Patient-centricity in labeling means that a low-risk patient with a limited medical history might receive a much shorter label, while a higher-risk patient with a history of cardiac issues would receive a detailed label, describing possible interactions with other medicines they may take, or other information related to their particular risk profile. A digital approach could provide targeted information most relevant to the patient whilst still ensuring that all the product details are readily available to the patient when they require further information and to meet applicable legal requirements. Quality improvement can be built into the process as well. For example, user feedback in labeling could be used to generate content based on what other similar patients have found most helpful, or most understandable in their day-to-day situation.

Beyond emphasizing a patient's particular health needs, digital labeling has other potential for patient-centricity. Instead of reading a black-and-white label from the side of a medication box or a PDF on a webpage, the label information could be presented audibly for

the visually-impaired, or in various font sizes and colors as needed. Digital labels could more readily include images and diagrams, and switch between multiple languages as well.

For drugs that are more complex to administer, such as combination products including medical devices, written instructions could be accompanied via an instructional video. A recent survey suggested that patients prefer a video link to be sent by their providers to outline drug protocols (Heath, 2019). Resources such as video tutorials can encourage a patient's willingness to adhere to their medications and have a visual interpersonal interaction. For example, personalized educational videos can help to teach end-users how to administer the medicine step-by-step. Linking such content to the label itself can potentially help improve drug adherence, and the odds that the therapy is successful.

### **A Means To Communicate With Patients**

Present-day drug labeling is a one-time, one-way communication from drug manufacturers to patients, and is most likely to be read at the time they first receive the medicine. However, even one-way communication can be enhanced with digital labels and modalities. In terms of the label itself, existing technology allows pharmaceutical companies and HCPs to push notifications to patients' mobile devices that highlight relevant changes in drug information. Such notifications could tell a patient when a product is nearing its expiry date, or when revised label information is available. Aside from labeling information, push notifications can deliver other content like a new and highly relevant study being published, resources to read more about a drug or disease, or connections to patient support and advocacy groups.

Beyond push notifications to patients, communication from the patients back to the pharmaceutical companies, HCPs and HAs could foster a valuable information feedback loop, leading to new insights about how patients interact with their labels. In a similar vein to what is commonly found on commercial products' frequently asked questions pages,

patients could share their opinion about a drug label's effectiveness by identifying particular sections as either more helpful or more challenging to understand. Patient feedback could also indicate the frequency of their referral to a label, and what information they sought out.

With comprehensive patient feedback, a digital label could point the user to helpful resources and drug information based on their personal needs and concerns. For example, if a patient is spending time on a drug's contraindications, this may suggest the patient needs more information or patient friendly languages.

### **Benefits**

The shift to patient-centricity and improved communication with patients has the potential to improve adherence to drug regimens, and ultimately, lead to better patient outcomes. Furthermore, a change in how drugs are labeled, and how drug information is provided, can potentially have wide-reaching impacts, extending far beyond the individual patient. As digital labels continue to evolve in response to patient feedback, the knowledge gathered can be redeployed to further enhance the information conveyed in labels.

### **Improved Patient Health**

Greater understanding of drug label information could empower patients to make informed decisions about how they take their medicines and manage their disease, all of which can be enhanced through a digital approach. A straightforward use case for digital labels is their improved ability to search for definitions, or to refer users to other information sources.

The many permutations of patient experience lend themselves to a customized, digital approach. A digital label that draws from personal health or demographic information can provide a better picture of how a drug will affect a particular patient, both in terms of its benefits and drawbacks. Dynamic digital labeling can take things a step further by helping patients know how to safely follow instructions related to dosing or how their drug relates to concomitant medicine use, and overall support them in better managing their health and care.

### **Improved Public Health And Reduced Health Care Spending**

Digital labeling has the unique feature of being able to actively collect data on its usage by its patients. By collecting, aggregating, and analyzing patient experience data with HCPs, industry and regulators, digital labeling can provide previously unknown insights into their patients populations.

Data derived from patient experience such as the uptake of labeling information, or patient perceptions around taking the product, contains valuable insights into the health literacy of different populations. These insights can help ensure that no patient group misses out on the potential of a therapy because of a misunderstanding.

Prevention is equally important from the individual perspective as well. Large-scale changes driven by patient insights would mean that patients everywhere could have far-superior access to drug information than is common today. Better-informed patients can make upstream changes to better manage their health, leading to reduced health care utilization, lower costs, and ultimately, improved quality of life.

### **Risks**

There is clearly huge opportunity with the digital labeling paradigm, but what risks need to be considered as part of future development? Firstly, every patient is different, and socio-economic, cultural and behavioural approaches to the use of digital technology vary tremendously. In the move to digital, it is essential that no patient is left behind and experiences a worse situation than currently exists. Furthermore, MAHs must continue to ensure compliance with applicable legal requirements, for example in relation to the information that can be provided to patients, and in terms of pharmacovigilance, as this transition to a digital labeling environment occurs.

While establishment of global principles and standards underpinning digital labeling would certainly drive its development, appropriate flexibility could be left for national authorities to consider the local environment. A range of hybrid approaches can solve most of these issues – for example just-in-time

printing of the latest label at the pharmacy, and/or a range of different paper labels (eg pictorial) to service different health literacy levels may be appropriate.

Connection with electronic health records and the management of patient data needs to be highly secure, and confidential in line with data protection laws and regulations. Product information also needs to be available offline when internet access is unavailable.

These are not small challenges to surmount, but the availability of digital technologies today and the rapid emergence of standards and interoperability, suggest that a path to the future is getting increasingly clear.

### What's Needed To Make Digital Labels A Reality

Despite the many benefits of a switch to digital labeling, it is important to be realistic about the challenges ahead. Several factors must first be addressed before this new paradigm can take root and reach its full potential. Realistically, the shift to digital labels will be a gradual process, and whilst all the benefits described in this paper will not necessarily be realized from the outset, this does allow for early experiences to offer a foundation for future development.

**Regulatory Considerations:** Before wholesale changes to labeling are possible, there are significant regulatory challenges to overcome in each market, such as privacy, software-as-a-medical-device regulation, label structure and content requirements, and regulations related to direct-to-patient communication. Looking further ahead, as new issues arise, different regulators will respond to the same issues differently, requiring ongoing coordination to assure that digital labeling practices remain compliant with rules established and updated by various legal bodies across the globe.

**Alignment Of Perspectives:** A successful digital labeling implementation requires alignment across numerous and varying stakeholders including patients, HCPs, pharmaceutical companies, HAs, and more. The volume of stakeholders significantly increases when considering digital labels from a global perspective, and it will be important to recognize and address the full breadth of viewpoints

This alignment is critical to ensure that the digital end state can truly address existing unmet needs, and make a meaningful difference to patients.

#### Standardization Of Data Formats:

In addition to more general consensus-building on a global regulatory and marketing approach, the label data/content itself will need a degree of harmonization. Standardizing the data structure across stakeholders will be necessary to easily share information. For example, to create a personalized digital label, the drug manufacturer would need to integrate with the patient's electronic health records or other personal health data sources.

**Access And Adoption:** Despite the benefits of digital labels, it will be difficult to provide uniform access to technology and to ensure patients acceptance of this new approach to receive drug product information. For example, internet access in rural communities in emerging markets may not be prevalent, suggesting that digital labeling will continue to be extremely difficult to achieve in certain locales. Even where the internet is ubiquitous, digital adoption problems can occur. When the UK recently introduced digital label access to patients via QR codes, regulators and marketers found limited and highly uneven consumer adoption because the technology has not been widely adopted into many peoples' daily lives. These realities underscore the importance of ongoing improvements to make paper labels more accessible and effective while also implementing and improving digital labels.

**Ongoing Learning:** As with any change that has a direct impact on patient experiences and indirect impacts to health outcomes, it is critical that the change be closely monitored to ensure that early experiences gained can contribute to future development and optimization of the digital concepts. make sure unintended consequences can be detected early on, and addressed appropriately.

**Time And Resources:** Additional costs incurred by drug manufacturers and regulators could ultimately be transferred to patients. Even with government funding or other capital investments, digital labeling will be a significant undertaking. For that reason alone, it is essential for stakeholders to understand their value proposition.

### Conclusion

Today's paper paradigm for product information is in dire need of a shakeup. Despite a massive and continual effort on the part of regulators and manufacturers, the standard drug label may be of marginal value to many who rely on them. To change this paradigm, patients need to better hear the messages on drug labels, and also to be better heard by the authors themselves.

As the first and oftentimes last means of communication between pharmaceutical companies and patients, there is a clear and compelling case for digital drug labels. A reconceptualized drug label is an underappreciated opportunity to improve the pharmaceutical use experience, thereby improving patient health, public health, and a host of other benefits.

In the end, regulators may or may not address the rules around labeling in view of technological advances and increasing consumerism. Regardless of what they do, the pharmaceutical industry can do more to improve the labeling process today. Given the proven impacts that digitization and consumer feedback has had in many other industries, it is imperative that the pharmaceutical industry give its own communications, and its product labeling, a fresh look. To do otherwise is to fall short of our mission to improve lives. ❖❖

IV124325

#### About the Authors:

*Amy Cheung is a Principal in Deloitte Consulting's Life Sciences practice specializing in R&D.*

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*Mukesh Singhal is a Senior Manager in Deloitte Consulting's Life Sciences R&D practice with over 15 years of experience within the industry.*

*Shimon Yoshida is Executive Director at Pfizer, and heads the International Labeling Group in Global Regulatory Affairs.*

*Sharon Gorman is Director of Global Regulatory Policy and Intelligence for Pfizer and responsible for the development and implementation of strategic policy initiatives.*

# The Case for Agile Innovation In Health Care



In a recent Bain & Company survey, nearly 80% of health care executives said they needed to be more agile. Yet more than half said they were not familiar with formal agile methodologies and tools, and were not using them in their companies.

**BY KALYAN JONNALAGADDA,  
DAVE FLEISCH, PETE HULTMAN  
AND STEVE BEREZ**

Health care and life science companies face mounting pressures to innovate faster in product development, services and customer experience.

In many industries that thrive on innovation, agile management already has transformed the way organizations work, helping small teams solve complex problems rapidly and deliver breakthrough results.

Now leading health care and life science executives are turning to agile teams to unleash a powerful burst of innovation.

In a recent Bain & Company survey, nearly 80% of health care executives said they needed to be more agile. Yet more than half said they were not familiar with formal agile methodologies and tools, and were not using them in their companies. Only 30% of respondents reported that their teams agreed on what it means to be agile (see *Exhibit 1*). So, although health care executives are determined to adopt agile management, most lack the methodology and even the language to implement it in practice.

Agile innovation is a set of methodologies – “Scrum” being the most common – that represent a faster, more efficient and collaborative way to work. Agile teams are small, self-governing, cross-functional groups dedicated to solving complex problems in a rapid fashion. They work closely with customers, developing a series of prototypes to speed innovation in products, services and processes. Their primary goal: delighting the customer.

The tumultuous changes in health care make a powerful case for agile innovation. More than 60% of the 321 health care and life science executives surveyed said time to market is increasingly important; a number that spiked to nearly 70% among executives at medical device companies. Another major shift: 60% of health care executives said that over the next three years companies will need to adapt rapidly to evolving customer needs; only 38% said that rapid adaptation was necessary during the past three years (see *Exhibit 2*). As digital natives, including Amazon, challenge traditional health care companies, the ability to react swiftly will become even more critical.

Innovation in services and customer experience increasingly determines whether health care teams win a sale. Bain research in multiple therapeutic areas has shown that when physicians prescribe a drug, the efficacy, safety and side effect profile of the drug account for only 50% to 60% of the physician’s choice—and that figure has declined over time. The other 40% to 50% is based on a range of physician and patient experience

factors that leading companies target to differentiate their products.

Looking at the past three years, only 8% of health care executives agreed that the best product no longer wins; instead, innovating in the services, information and customer experience around the product is critical. That number jumps almost fourfold, to 28%, when executives look ahead to the next three years (see Exhibit 3). The product matters, but other innovations are increasingly important. Customer needs evolve continually, and leading companies respond rapidly to changing market demand.

Initially, some of the core agile methods, such as rapid prototyping with customers and test and learn, seemed at odds with the highly regulated development processes for drugs and medical devices. Health care executives, however, are taking a broader view of when and where to use agile innovation. In addition to research and development, they are looking at services, customer experiences and internal processes that shape those experiences. The payoff in results and performance can be huge. Health care companies that have begun deploying agile teams report a step change in those teams' performance. Seventy-five percent of executives at these companies told us that their agile teams perform better or significantly better than traditional teams do.

### A New Universe Of Customers

Agile innovations can target patients, insured members, physicians and even internal processes. Here are examples of success stories from agile teams.

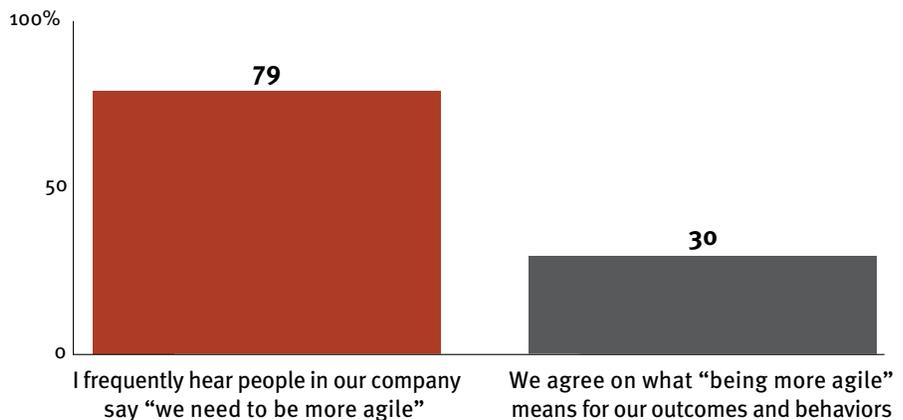
#### Patient experience in biopharma.

While high failure rates in drug development are well known, pharma companies also have a remarkably high failure rate in service innovation. Our panel reports that 65% of service innovations fall short of expectations. That is a sweet spot for agile innovation. Leading companies deploy agile teams that work closely with customers to get service innovations right.

Take the case of a major biopharma company that set out to improve the patient experience with several key products. Senior executives created an agile brand team for a drug that had been on the market for a decade in a highly competitive therapeutic area. The team gleaned important new insights through weekly discussions with a panel of 30 patients.

### Exhibit 1 Most Do Not Agree On What Agile Means

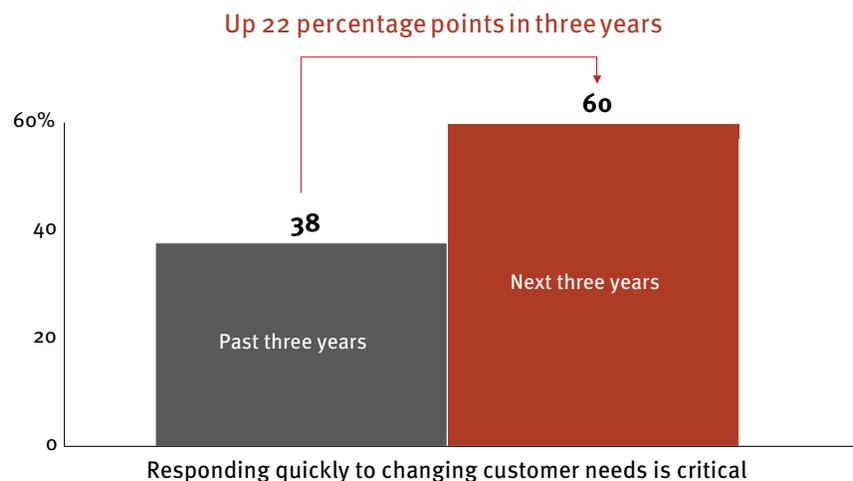
Percentage of respondents who agree or strongly agree



Note: Survey question—thinking about the use of the terms agile/agility and the agile methodology within your organization, to what extent do you agree with the following statements? (N=321)

### Exhibit 2 Executives Recognize Need To Respond To Changing Customer Needs

Percentage of respondents who strongly agree



Note: Survey question—looking back at the past three years/ahead at the next three years, to what extent do you agree with the following statements? (N=321)

In particular, patients had difficulty understanding the complex steps involved in starting treatment. The agile team worked rapidly to prototype a series of improvements to better guide patients through the early stages of treatment. Within four months, the company had tested the new solutions and introduced them to the market, significantly improving the customer experience. The team is now using patient feedback continually to improve its solutions and tackle a new set of opportunities that the patients identified.

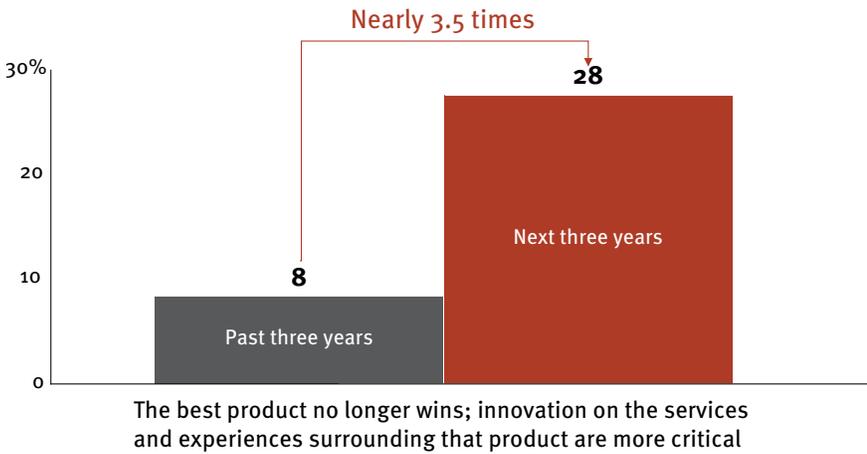
The same company created another agile

team to enhance the physician experience with its products, leading to improved customer engagement and the development of digital solutions. The multidisciplinary team also cut the lead times for developing prototypes and conducting initial customer tests by as much as 90% (see Exhibit 4).

**Payer product innovation.** Leading payers are deploying agile teams in core product innovation, care management, patient engagement, provider engagement, clinical operations and medical cost management. A large US payer-provider turned to agile after several efforts to im-

### Exhibit 3 Having The Best Product Is No Longer Enough

Percentage of respondents who strongly agree



Note: Survey question—looking back at the past three years/ahead at the next three years, to what extent do you agree with the following statements? (N=321)

prove medical cost management were slow to gain traction. Senior executives bet that agile’s cross-functional teams would improve collaboration between business units and functions, as well as provide more effective leadership. The company launched a pilot program in four key areas:

One agile team working on improving management of high-cost patients developed an analytics tool to identify these potential individuals early on. The team, which included physicians, case and care managers, and data analytics experts, created a patient-routing algorithm to match the individual with a dedicated team of nurses, doctors and specialists.

That allowed the payer-provider to arrange support and treatment before costs started rising. The success of the four pilot teams convinced the company to set up an agile center of excellence to extend the practice to other parts of the organization.

**Accelerating enrollment of clinical trials.** Quickly enrolling patients in clinical trials is a key challenge for pharma and medical device companies eager to speed new products to market. In competitive markets, faster enrollment increases the product’s commercial potential substantially. One leading biopharma company observed a large difference in patient enrollment rates across its clinical trial sites. The

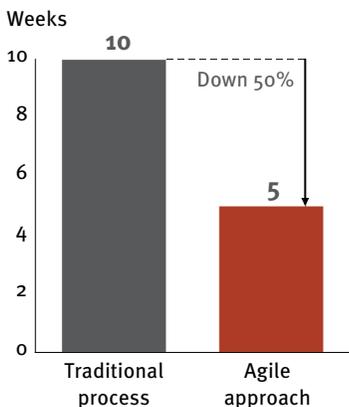
study team at some sites felt confident after being trained and were ready to enroll their first patient. Other sites found the training to be hard to remember and access later. The study teams had divergent responses on other important issues, too, including the site team’s ability to communicate effectively with prospective patients and the quality and volume of patient referrals each site received. The clinical trial sites in which investigators reported positive experiences were enrolling twice as many patients in the trials as those sites in which investigators were neutral or unhappy.

The company used agile teams to launch a series of innovations in site start-up and enrollment support to improve investigators’ experiences. They tested new ideas and prototypes every two to three weeks with a panel of customers that included investigators and their site teams. Six weeks after Agile teams went to work, the company launched a new training program for the clinical trial sites. Every few weeks, it rolled out additional innovations that removed obstacles for the site teams, accelerated the pace of enrollment and improved referral quality, among other gains. The response from the trial sites has been so positive that the company is now beginning to extend these practices to its entire portfolio of clinical trials.

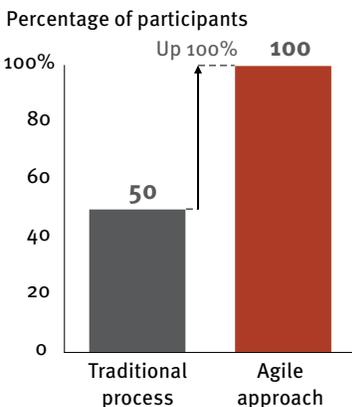
**Pricing in medical devices.** Health care companies are also making strides in internal process innovation. One leading medical devices company found its pricing to be inconsistent with its customer segments.

### Exhibit 4 Agile Teams Significantly Accelerate Customer Solutions

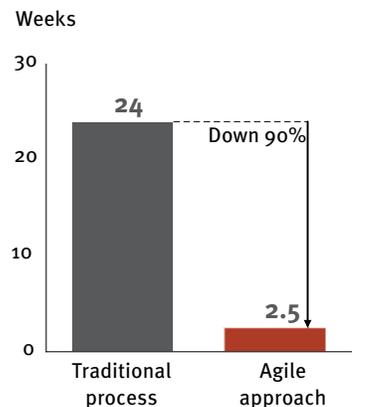
Prepared and ran solution design workshop with oncologists



Signed up workshop participants for follow-up engagements



Developed digital prototype, and conducted user test



On closer examination, executives realized that product prices varied significantly, but no one could explain why. The company deployed agile teams to improve customer segmentation, pricing guidance, approval processes, tools, reporting and financial goals. In each area, the teams tackled small changes and innovations, testing prototypes with sales operations and sales management, making adjustments based on feedback, and then moving on to the next small adaptation. The company's financial performance began to improve after six weeks, and the leadership team eventually introduced similar changes to core pricing systems and processes throughout the company.

### Good Agile Versus. Bad Agile

By far, the biggest impediment to using agile teams successfully is a failure to adhere to the basic principles and methods. We call that approach "bad agile." If you hear a colleague saying, "We want to be agile, but we're not going to adopt agile innovation (or Scrum or other agile methodologies)," that's a signal that things are likely to go wrong. The reason is clear: when companies use the language of agile innovation without changing how they work, their underlying agility tends to stay the same. When we asked our 321 senior health care executives about recent innovation failures in their companies, the three most cited reasons were ineffective cross-functional collaboration, failure to collaborate effectively with customers and failure to dedicate sufficient resources to scale projects. Agile solves each of these problems.

Yet some health care executives may worry about delegating decision making to their teams or that focusing on customer demands reduces attention to financial

performance. Companies can overcome these obstacles by initially deploying agile teams to a few high-priority areas with leaders open to experimentation. That approach helps demonstrate the benefits to skeptical colleagues while also addressing their concerns.

Others resist agile methodologies because the change is not easy. Agile teams are more effective than traditional teams, and their morale is significantly higher. But it is a discipline that takes work. Managers must learn a different approach to forming teams and interacting with them. When companies commit to that approach, they are more successful. Yet many fall short. In our survey, 60% of those who have used agile innovation said they underinvested in teaching and coaching their senior leaders on how to best interact with and enable their agile teams. That slowed the benefits. On the other hand, nearly half of the respondents using agile teams appointed dedicated product owners. In these companies, agile teams felt more capable of making day-to-day decisions.

### Agile At Scale

Once companies have deployed a few agile teams effectively, the next step is extending agile innovation across large parts of the organization. Scaling agile innovation requires executives and the broader management team to change some practices. For example, managers need to focus on rapidly removing barriers for their teams as well as adopting methods to manage interdependencies among agile teams, and between those teams and other parts of the organization. Over time, scaling agile practices also changes resource allocation and how companies evaluate and reward teams. Only 23% of executives experienced with agile agreed that "our

planning, budgeting and resource allocation processes can shift resources quickly (enough) today," and only 34% said that "my company's culture naturally enables agile." (See Exhibit 5.)

But when companies have built the appropriate foundation, agile practices can transform the way they innovate. One large health care organization used a multiyear agile program to accelerate innovation across the entire organization. Senior executives took a holistic approach that changed leadership behaviors, organizational culture, and the company's software development process and funding model, significantly improving speed to market.

### A New Era

Health care companies are under increasing pressure to improve quality and reduce costs. To win in the coming decade, leaders are reinventing their organizations for rapidly evolving markets. Above all, they are innovating faster and designing better customer experiences. Agile teams allow these companies to focus quickly and intensively on the set of innovations that matter most, to get prototypes in front of customers, and to rapidly test, learn and adapt. Leadership teams that seize those advantages will have the best shot at staying on top in a fast-changing landscape. ❖

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*About The Authors: Kalyan Jonnalagadda, Dave Fleisch, Pete Hultman and Steve Berez are partners with Bain & Company's Healthcare practice. Kalyan Jonnalagadda and Dave Fleisch are based in Bain's New York office, Pete Hultman is located in the company's San Francisco office, and Steve Berez is based in the Boston office.*

SOURCE FOR ALL EXHIBITS: Bain & Company

## Exhibit 5 Most Companies Struggle With Four Main Obstacles



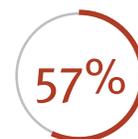
Misaligned funding model for agile



Lack of an enterprise-wide strategy for agile



Company culture that does not support agile



Missing key IT enablers for agile software teams

Note: Survey question—thinking about the specific situations in which agile methods have been used at your company, to what extent do you agree with the following statements? (N=321; n=21 for IT respondents)

# On the Move

Recent executive appointments  
in the life sciences industry



■ SOREN BREGENHOLT



■ HELEN GIZA



■ JASON MEYENBURG



■ SHAKTI NARAYAN

## COMPANY CHANGES

EXECUTIVE	TO COMPANY	NEW ROLE	FROM COMPANY	PREVIOUS ROLE	EFFECTIVE DATE
Shakti Narayan	Accent Therapeutics Inc	Chief Executive Officer and Director	Tango Therapeutics	Chief Business Officer	3-Sep-19
Ralph Halter	Acino International AG	Global Head, IT	Panalpina	Corporate Head, IT Strategy and Planning	1-Oct-19
Gregory Robinson	Akouos	Chief Scientific Officer	Nightstar	Chief Scientific Officer	4-Sep-19
Mark Sardi	Ascendis Health	Chief Executive Officer	House of Busby	Chief Executive Officer	14-Oct-19
Daniella Foster	Bayer AG	Head, Public Affairs and Sustainability, Consumer Health Division	Hilton Corp	Leader, Global Corporate Responsibility	16-Sep-19
Marianne De Backer	Bayer AG	Head, Business Development and Licensing and Executive Committee Member	Johnson & Johnson	Head, Business Development	3-Sep-19
Patrice Ferrand	Biom'Up SA	Chief Executive Officer and Chairman, Biom'up USA Inc	Unilabs France	Chief Executive Officer	18-Sep-19
Preetam Shah	BrainStorm Cell Therapeutics Inc	Chief Financial Officer and Executive Vice President	Barclays Capital plc	Director, Healthcare Investment Banking	9-Sep-19
Jeffrey A. Munsie	Concert Pharmaceuticals Inc	Chief Legal Officer	Merrimack Pharmaceuticals	Senior Vice President, General Counsel and Head, Corporate Operations	23-Sep-19
David Garrett	Dynacure SAS	Chief Financial Officer	Nabriva Therapeutics	Vice President, Corporate Controller and Head, Investor Relations	3-Sep-19
Aurelie Grienerberger	Eligo Bioscience	Chief Business Officer	Sanofi	Director, Transactions, Global Licensing and Business Development	17-Sep-19

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■ **RICHARD RIESE**



■ **GREGORY ROBINSON**



■ **PATRICK TERRY**



■ **ROBERT WEISKOPF**

#### COMPANY CHANGES

EXECUTIVE	TO COMPANY	NEW ROLE	FROM COMPANY	PREVIOUS ROLE	EFFECTIVE DATE
Helen Giza	Fresenius Medical Care	Chief Financial Officer	Takeda Pharmaceuticals	Chief Integration and Divestiture Management Officer	1-Nov-19
Jason Meyenburg	Gemini Therapeutics	Chief Executive Officer and Director	Orchard Therapeutics	Chief Commercial Officer	13-Sep-19
Jack Hu	Genor BioPharma Co Ltd	Chief Financial Officer and Chief Strategy Officer	Deutsche Bank	Managing Director and Head, APAC Healthcare Research	3-Sep-19
Catherine Michel	Halma plc	Chief Technology Officer	Sigma Systems	Chief Technology Officer and Chief Strategy Officer	16-Sep-19
Jacquelyn Fahey Sandell	Jounce Therapeutics Inc	Chief Legal Officer and Corporate Secretary	Vericel Corp	Vice President, General Counsel and Corporate Secretary	17-Sep-19
Kathie M. Bishop	Locana Inc	Chief Scientific Officer	Otonomy	Chief Scientific Officer	4-Sep-19
Soren Bregenholt	Macrophage Pharma	Chief Executive Officer	Medicon Valley Alliance	Chairman	3-Sep-19
Amit Hasija	Milestone Pharmaceuticals Inc	Chief Financial Officer and Executive Vice President, Corporate Development	Fulcrum Therapeutics	Chief Financial Officer and Chief Business Officer	10-Sep-19
Tonya Saffer	Outset Medical Inc	Head, Government Affairs and Market Access	National Kidney Foundation	Vice President, Health Policy	24-Sep-19
John Snisarenko	Oyster Point Pharma Inc	Chief Commercial Officer	Shire	Group Vice President, Ophthalmic	19-Sep-19
Hitto Kaufmann	Pieris Pharmaceuticals Inc	Chief Scientific Officer and Senior Vice President	Sanofi	Head, Strategy and Operations	3-Sep-19

**COMPANY CHANGES**

EXECUTIVE	TO COMPANY	NEW ROLE	FROM COMPANY	PREVIOUS ROLE	EFFECTIVE DATE
Manuel O. Mendez	Quest Diagnostics	Chief Commercial Officer and Senior Vice President	Qiagen NV	Senior Vice President, Head, Global Commercial Operations	7-Oct-19
Robert Wasserman	Rgenix Inc	Chief Medical Officer	Northern Biologics	Chief Medical Officer	16-Sep-19
Patrick Terry	SomaLogic Inc	Chief Commercial Officer	MeMed Diagnostics	Director	10-Sep-19
Robert Weiskopf	Stealth BioTherapeutics Inc	Chief Financial Officer	ArQule	Chief Financial Officer and Treasurer	3-Sep-19
Andrew Parker	Step Pharma	Chief Executive Officer and Supervisory Board Member	Zealand Pharma	Chief Science Officer and Executive Vice President	6-Sep-19
Richard Riese	Synlogic	Chief Medical Officer	Alnylam	Vice President, Clinical Development	4-Sep-19
Elcin Barker Ergun	The Menarini Group	Chief Executive Officer	Merck KGaA	Head, New Business	12-Sep-19
Jan Skvarka	Trillium Therapeutics Inc	Chief Executive Officer, President and Director	Tal Medical	Chief Executive Officer and President	25-Sep-19
Francis R. Facchini	Varian Medical Systems Inc	Chief Medical Officer, Interventional Oncology	BTG plc	Chief Medical Officer and Head, Medical Affairs	16-Sep-19

**PROMOTIONS**

EXECUTIVE	TO COMPANY	NEW ROLE	PREVIOUS ROLE	EFFECTIVE DATE
Jason Barker	Arcus Biosciences	Principal Financial Officer and Principal Accounting Officer	Vice President, Finance	5-Sep-19
Thomas E. Polen	Becton, Dickinson and Co	Chief Executive Officer and President	Chief Operating Officer and President	28-Jan-20
George Makhoul	Biom'up USA Inc	Chief Executive Officer	Chief Commercial Officer	18-Sep-19
Bill Steinkrauss	Curis Inc	Chief Financial Officer	Treasurer and Vice President, Finance	12-Sep-19
Quentin Blackford	DexCom Inc	Chief Operating Officer and Chief Financial Officer	Chief Financial Officer	17-Sep-19
Patrick Kelly	FORMA Therapeutics	Chief Medical Officer and Senior Vice President	Vice President, Translational Medicine	24-Sep-19
Pascal Prigent	GENFIT	Chief Executive Officer	Executive Vice President, Marketing and Commercial Development	2-Sep-19
Suneil Hosmane	GENFIT	Head, Global Diagnostics	Executive Vice President, Diagnostics	26-Sep-19
Gitte Pugholm Aabo	GN Store Nord AS	Chief Executive Officer, GN Hearing	Director	20-Sep-19
Nadir Mahmood	Nkarta Therapeutics	Chief Business Officer	Senior Vice President, Corporate Development	18-Sep-19
Begona Carreno	Novartis AG	Global Business Development and Licensing Head, Ophthalmology	Global Business and Licensing Head, Respiratory	27-Sep-19

## PROMOTIONS

EXECUTIVE	TO COMPANY	NEW ROLE	PREVIOUS ROLE	EFFECTIVE DATE
Beatrice Clerc	Novo Nordisk Canada Inc	President	Corporate Vice President and General Manager, Novo Nordisk France	13-Sep-19
Jennifer Kosharek	Nuvectra	Chief Financial Officer	Interim Chief Financial Officer	16-Sep-19
Prahlad Singh	PerkinElmer Inc	Chief Executive Officer	Chief Operating Officer and President	30-Dec-19
David Thomson	Precision BioSciences Inc	Chief Operating Officer	Chief Development Officer	23-Sep-19
Kimberly Peery	Stereotaxis	Chief Financial Officer	Vice President, Finance	1-Oct-19
Desmond Hirson	Ventripoint Diagnostics Ltd	Chief Technology Officer	President	19-Sep-19

## DIRECTORS

EXECUTIVE	TO COMPANY	NEW ROLE	EFFECTIVE DATE
David L. Mahoney	Adamas Pharmaceuticals Inc	Chairman	16-Sep-19
Maarten de Chateau	Beactica AB	Director	10-Sep-19
Bridgette Heller	DexCom Inc	Director	17-Sep-19
Marianne Kirkegaard	Fertin Pharma AS	Chairman	1-Sep-19
Elaine V. Jones	Gritstone Oncology Inc	Director	12-Sep-19
Julie Beck	Invacare Corp	Director, Member, Audit, Nominating and Governance Committees	18-Sep-19
Colleen F. Reitan	Myriad Genetics Inc	Director	25-Sep-19
Lee N. Newcomer	Myriad Genetics Inc	Director	25-Sep-19
Peter Cohen	PolarityTE Inc	Chairman	4-Sep-19
Debora Spar	Thermo Fisher Scientific Inc	Director	5-Sep-19
Lota Zoth	Zymeworks Inc	Chairman	17-Sep-19

## ADVISORS

EXECUTIVE	TO COMPANY	NEW ROLE	EFFECTIVE DATE
Ryan B. Corcoran	C4 Therapeutics	Scientific Advisory Board Member	23-Sep-19
Samir Awad	Endonovo Therapeutics Inc	Scientific Advisory Board Member	9-Sep-19
Rizwan Romee	Glycostem	Advisory Board Member	12-Sep-19
E. Antonio (Nino) Chiocca	Immunomic Therapeutics Inc	Scientific Advisory Board Member	10-Sep-19
Stephen Shuttleworth	Karus Therapeutics Ltd	Chairman, Scientific Advisory Board	17-Sep-19
Rodrigo Bianchi	Limacorporate SpA	Advisory Board Member	18-Sep-19
Carlos Perez	Mevion Medical Systems Inc	Senior Advisory Committee Member	11-Sep-19
Derrick Chambers	Qrons Inc	Advisory Board Member	23-Sep-19
Frank Gerberick	RenovaCare Inc	Advisor	24-Sep-19

# Deal-Making

Covering deals made September 2019

Derived from Strategic Transactions, Informa's premium source for tracking life sciences deal activity, the Deal-Making column is a survey of recent health care transactions listed by relevant industry segment – In Vitro Diagnostics, Medical Devices, Pharmaceuticals, and Research, Analytical Equipment and Supplies – and then categorized by type – Acquisition, Alliance, or Financing.

Strategic Transactions is updated daily with in-depth deal analysis, structural and financial terms, and links to SEC-filed contracts.

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Curetis, OpGen merge

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GSK buys Sitari for undisclosed sum

Lundbeck acquires Alder BioPharmaceuticals for up to \$20/share, a potential \$1.95bn

Swedish Orphan buys Dova for \$915m

Vertex pays \$950m for diabetes-focused Semma

Investment firm PAG pays \$540m for majority ownership of Hisun BioRay

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Amerigen licenses US rights to two Inventia approved ANDAs

Amplyx licenses global rights to Novartis' shelved MAU868 for BKV disease

Atomwise, Hansoh collaborate on small-molecule drug discovery

BMS becomes limited partner in BioMotiv, holds options to acquire investments

BI, Inflammasome ally in \$160m retinal diseases collaboration

BI gains worldwide rights to Lupin's LNP3794 for GI and lung cancers

Oncoheroes gains worldwide license to BI's shelved volasertib cancer compound

Mundipharma gets license to Cidara's antifungal rezafungin

Wize gets ophthalmic IP from Copernicus

Curax gains NA license to OptiNose's Onzetra Xsail migraine treatment

Daiichi Sankyo gains Brazilian rights, other options on Mitsubishi Tanabe's edaravone for ALS

Evotec and Takeda partner in deal worth over \$850m

Flexion licenses Xenon's XEN402 for post-operative pain

GSK, VBI enter cancer trial collaboration

Grunenthal gains license in certain markets to Mesoblast's MPCo61D cell therapy for low back pain

Novartis funds research collaboration with IFM Due, gains option to acquire company outright

Tris gets US license to second CNS generic from Intellipharmaceutics

Simcere gets Chinese rights to JW's Phase IIb gout compound

KemPharm licenses ADHD compounds to VC firm Gurnet Point Capital

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Acadia nets \$271.7m through FOPO

Aerie announces upsized offering of \$275m notes

Upon close of ArTara/Proteon merger, investors to provide \$42.5m in funding

Bellus Health nets \$73.8m in US IPO

BioNTech files for initial public offering

Public offering nets \$18.8m for Celyad

Fate Therapeutics nets \$141.5m through public offering

**Frequency Therapeutics** files for IPO  
IPO nets \$187m for **IGM Biosciences**

**OptiNose** enters debt facility with Pharmakon

**Plus Therapeutics** nets \$13.9m via public offering

Public offering nets \$37.6m for **Provention Bio**

Private placement from **Amgen** grosses \$20m for **Provention**

**PTC Therapeutics** sells \$250m in convertible debt financing

**PTC Therapeutics** nets \$97.3m via FOPO

**Satsuma** nets \$76.7m in IPO

**SpringWorks** nets \$173.3m via IPO

**Translate Bio** nets \$84.6m via FOPO

**Turning Point** public offering nets \$190m just months after IPO

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IPO nets \$417m for **10x Genomics**

## IN VITRO DIAGNOSTICS

### MERGERS & ACQUISITIONS

#### OPGEN INC. CURETIS NV

**Curetis NV** and fellow public infectious disease-focused molecular diagnostics firm **OpGen Inc.** are reverse merging. (Sep.)

Curetis will receive 2.66m OpGen shares (representing 72.5% of the firm), valuing the combined business at \$24m. The combined entity will retain the OpGen name and have its headquarters in the US at OpGen's Maryland offices, while European operations will continue at Curetis' German facilities. Curetis' **Ares Genetics** subsidiary will continue offering bioinformatics and next-generation sequencing (NGS) service laboratory operations in Austria. Curetis' current CEO Oliver Schacht, PhD, will continue in that role in the merged entity and OpGen CFO Timothy Dec will retain his position. The board will be chaired by Curetis' William Rhodes and have representation from four members of Curetis and two from OpGen. OpGen's *Acuitas Lighthouse* is the first cloud-based software that can identify, track, and predict antibiotic-resistant infections based on genotypes and phenotypes. The company also offers the *AdvanDx* tests for the fast and accurate identification of bacteria and yeast directly from a patient's positive blood cultures. Its *QuickFISH* and *PNA FISH* tests use peptide nucleic acid fluorescence in situ hybridization (PNA FISH) technology and provide results in just 20 minutes, whereas conventional methods can take up to 72 hours. As a result, patients have a shorter hospital stay, thus lower costs while avoiding the need for broad-spectrum antibiotics. Curetis' portfolio includes the *Unyvero* platform that uses multiplex PCR technology to simultaneously and rapidly detect a wide variety of microorganisms, antibiotic resistance markers, or toxins from one sample using a disposable cartridge. The merged entity will focus on rapid diagnostics for various infections including lower respiratory and urinary tract infections and pneumonia, as well as antimicrobial resistance. Investment Banks/Advisors: Crosstree Capital Partners (OpGen Inc.); HC Wainwright & Co. (Curetis NV)

### FINANCINGS

#### CHEMBIO DIAGNOSTICS INC.

**Chembio Diagnostics Inc.** (infectious disease tests) entered into a \$20m four-year term loan with an affiliate of Perceptive Advisors. The loan bears interest at an annual rate of 8.75% plus the greater of one-month LIBOR and 2.5% and requires no principal payments during the first three years. Chembio also issued to Perceptive Advisors a seven-year warrant to purchase 550k shares at an exercise price of \$5.22. The company will use the proceeds for R&D, commercial activities, and to expand its US facilities and automate manufacturing. (Sep.)

Investment Banks/Advisors: Craig-Hallum Inc.

#### HTG MOLECULAR DIAGNOSTICS INC.

**HTG Molecular Diagnostics Inc.** (next-gen sequencing-based molecular profiling) netted \$17.9m through a public offering of 29.3 million common shares (including the over allotment) at \$0.65. Certain institutional investors also purchased 5.4 million pre-funded warrants (exercisable for 5.4 million common) at \$0.64 piece for additional net proceeds of \$3.3m. (Sep.)

Investment Banks/Advisors: Cantor Fitzgerald & Co.

#### INVITAE CORP.

Genetic testing firm **Invitae Corp.** netted \$291.2m through an upsized private offering of \$300m aggregate principle amount of 2% senior notes due 2024. The notes convert to common at a rate of 33.6293 shares per \$1k principal amount, or \$29.74 per share. (Invitae's stock averaged \$24.46 at the time of the transaction.) **Invitae** originally planned to issue \$200m of the notes. (Sep.)

## MEDICAL DEVICES

### MERGERS & ACQUISITIONS

#### BAXTER INTERNATIONAL INC. CHEETAH MEDICAL INC.

**Baxter International Inc.** will acquire privately held monitoring device firm **Cheetah Medical** for \$190m in cash plus up to \$40m in earn-outs based on clinical and commercial achievements. (Sep.)

Cheetah was founded in 2000 and designs and markets specialized noninvasive hemodynamic monitoring systems. Through the acquisition, Baxter gains the company's *Starling SV* system, which provides a dynamic assessment of fluid responsiveness for accurate maintenance of organ and tissue perfusion. The technology is based on Cheetah's *Bioreactance* technology which uses the application of electrical current (AC current) through external sensors applied at the thorax to help measure time delay and phase shifts, parameters which are tightly correlated with cardiac stroke volume. Baxter notes that the Cheetah platform will be integrated with its own IV infusion pumps, IV fluid offerings, and medications to provide a more integrated patient monitoring and treatment experience.

#### INTEGRA LIFESCIENCES HOLDINGS CORP. REBOUND THERAPEUTICS CORP.

**Integra LifeSciences Holdings Corp.** acquired privately held minimally invasive surgery (MIS) technologies start-up **Rebound Therapeutics Corp.** (Sep.)

Rebound makes single-use medical devices that enable imaging during minimally invasive neurosurgeries and other MIS procedures, providing access and using integrated optics and illumination for visualization. Rebound's *Aurora* surgiscope system and evacuator were both FDA cleared earlier this year. The *Aurora* surgiscope consists of a disposable neurosurgical endoscope and a reusable control unit that enables the user to control a high-definition video image of the neuro anatomy. The *Aurora* evacuator is an electrosurgical device that includes a power instrument and a single-use handpiece for soft tissue aspiration or removal in neuro, spinal, pelvic, ENT, and other MIS procedures, enabling direct visualization of surgical sites with limited access. Since its 2015 inception, Rebound has raised \$20.3m in venture funding. The addition of Rebound's products will enhance Integra's existing MIS neurosurgical instruments portfolio.

#### STRYKER CORP. CARDAN ROBOTICS MOBIUS IMAGING LLC

**Stryker Corp.** agreed to acquire privately held device companies **Mobius Imaging LLC** (point-of-care imaging) and its sister spinal robotics company, **Cardan Robotics** (aka GYS Tech LLC; navigation), in an all cash transaction. (Sep.)

Stryker will pay \$370m up front and could provide up to \$130m in earn-outs associated with the achievement of development and commercial milestones. Founded in 2008, Mobius creates tools that fit into and assist the imaging workflow. Its 510(k)-cleared *Airo TruCT* scanner is a mobile, real-time x-ray computed tomography

(CT) imaging system for use in emergency departments as well as numerous applications in spine, orthopedic, and neuro surgeries, thoracic procedures, and radiation oncology. Cardan, founded in 2015, offers the *Orion* line of robotic arms and guidance systems, image-guided navigation systems (and related hardware and software) used in surgical and interventional radiology procedures. The *Orion* surgical suite integrates with Mobius' *Airo TruCT* scanner, to offer a complete collection of imaging, navigation, and robotic products for spinal surgeries. The addition of Mobius and Cardan provides complementary offerings to Stryker's implant and navigation portfolio, particularly Stryker's *Mako* robotic orthopedic surgery platform. The deal also marks the entry of Stryker's spine division into the intra-operative imaging market and gives Stryker a better advantage over competitors such as **Medtronic** and **Zimmer Biomet**, which both already sell robotic surgery systems for the spine.

#### ALLIANCES

##### BAYER AG ONE DROP

**One Drop** partnered its digital health platform with **Bayer AG**, which will use the technology to provide bio-digital solutions in the areas of oncology, cardiovascular disease, and women's health. The deal is worth \$10m. (Sep.)

While Bayer will be applying One Drop's platform in multiple indications, One Drop has traditionally focused on diabetes management. Its digital health offering includes coaching programs (with certified diabetes educators) and a mobile app that tracks blood sugar, medications, food, and activity, and incorporates artificial intelligence-based behavioral recommendations. The company also provides test strips, glucose meter start kits, and lancets. In addition to signing the licensing agreement, Bayer also led a \$40m series B round for One Drop.

##### CLEARSIDE BIOMEDICAL INC. REGENXBIO INC.

**REGENXBIO Inc.** received an option to license exclusive global rights (including sublicensing rights) to **Clearside Biomedical Inc.**'s *SCS Microinjector*. (Sep.)

Should REGENXBIO exercise its option, it would pay Clearside a fee plus up to \$34m in development milestones and \$102m in sales milestones as well as mid-single-digit sales royalties. REGENXBIO would use the *SCS Microinjector* to non-surgically deliver its own RGX314 adeno-associated virus 8 (AAV8) gene therapy to the suprachoroidal space (SCS) for treating wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR), and other conditions for which anti-vascular

endothelial growth factor (anti-VEGF) treatment is currently the standard of care. The company would be responsible for all development and commercialization activities for gene therapy product candidates, while Clearside will supply the *SCS Microinjector*. RGX314 is based on REGENXBIO's NAV technology and is currently in a Phase I/IIa study for wet AMD; a Phase IIb clinical trial in wet AMD and a Phase II trial in DR are expected to begin by the end of the year. Clearside's *SCS Microinjector* enables the delivery of drugs to the SCS (the space between the sclera and choroid) in higher concentrations a lower dose.

#### FINANCINGS

##### DANAHER CORP.

*Envista Holdings Corp.*

**Danaher Corp.**'s dental subsidiary **Envista Holdings Corp.** (implants, orthodontics, and digital imaging technologies) netted \$643m in its initial public offering of 30.7 million shares (including the overallotment) at \$22, the middle of its anticipated \$21-24 range. (Sep.)

Investment Banks/Advisors: Bank of America Merrill Lynch; Credit Suisse Group; Evercore Partners; Goldman Sachs & Co.; JP Morgan & Co.; Jefferies & Co. Inc.; Morgan Stanley & Co.; Robert W. Baird & Co. Inc.; Stifel Nicolaus & Co. Inc.; William Blair & Co. (Envista Holdings Corp.)

##### INSULET CORP.

**Insulet Corp.** (developer of the *Omnipod* insulin management system) announced an upsized private placement of \$700m (net \$684.3m) aggregate principal amount of convertible senior notes due 2026. The notes bear interest at a rate of 0.375% and can be converted in cash, stock, or a combination. The conversion rate will initially be 4.4105 shares of common stock per \$1k principal amount of notes, or \$226.73 per Insulin share (its shares are currently averaging \$154.65). The company is also granting the initial purchasers an option to purchase another \$100m notes. Insulet announced it is entering into capped call transactions and will use some of the proceeds to fund those transactions. Additional funds will be used to repurchase \$225m aggregate principal amount of the outstanding 1.25% convertible senior notes due 2021. (Sep.)

##### ISTAR MEDICAL SA

Ophthalmic implants maker **iStar Medical SA** raised €40m (\$44m) in its series C financing led by Life Sciences Partners and GIMV, which was joined by new backers Earlybird and BNP Paribas Fortis Private Equity, and returning shareholders Capricorn Partners, Walloon Region Investment Fund, and Belgian Federal Investment Fund. The company will use the proceeds

for ongoing development of its *MINIject* as the company prepares to commercialize the device in the US and Europe. *MINIject* is designed to reduce intraocular pressure in glaucoma patients. (Sep.)

## PHARMACEUTICALS

### MERGERS & ACQUISITIONS

#### ANOKION SA KANYOS BIO INC.

Four years after spinning out the company, **Anokion SA** has now acquired **Kanyos Bio Inc.** Financial terms were not disclosed. (Sep.)

Kanyos was formed in 2015 and tasked with developing celiac disease and Type I diabetes candidates arising from a partnership between Anokion and **Astellas**. (Astellas had an option to buy Kanyos under terms of that deal.) Through the acquisition, Anokion gains KAN101, an antigen-specific celiac disease therapy for which an IND filing is expected by the end of 2019. The project will be developed alongside Anokion's ANK780, also an antigen-specific treatment that is in preclinical studies for multiple sclerosis. Concurrent with the Kanyos buy, Anokion announced a \$40m Series B round.

#### ARTARA THERAPEUTICS INC. PROTEON THERAPEUTICS INC.

Publicly traded **Proteon Therapeutics Inc.** is reverse merging with closely held **ArTara Therapeutics Inc.** in a stock swap. (Sep.) Post-transaction, the combined firm will keep the ArTara name, trade on the Nasdaq under the ticker TARA, have its headquarters in New York City, and be led by currently ArTara CEO Jesse Shefferman. Mr. Shefferman will serve on the board along with five members designated by ArTara and one by Proteon. The company will be owned 90%/10% by ArTara and Proteon, respectively. The combined entity will focus on advancing ArTara's two assets. Lead program TARA002 (follow-on biologic of OK432 (picibanil), an attenuated strain of *Streptococcus pyogenes*) is a Phase III toll-like receptor 4 agonist for treating lymphangiomas. ArTara eventually plans to study the therapy for various other indications for which OK432 has shown potential. ArTara's second program is Phase IIa IV choline chloride for intestinal failure associated liver disease. Both compounds have orphan drug status in the US. A group of investors have agreed to invest \$42.5m in the combined company to fund development of TARA002 and IV choline chloride. Investment Banks/Advisors: HC Wainwright & Co. (Proteon Therapeutics Inc.); Ladenburg Thalmann & Co. Inc. (ArTara Therapeutics Inc.)

#### CASTLE CREEK PHARMACEUTICALS LLC FIBROCELL SCIENCE INC.

**Castle Creek Pharmaceuticals LLC** (CCP) agreed to acquire its partner, public US biotech **Fibrocell Science Inc.** (autologous cell-based therapies for skin and connective tissue diseases) for \$3 in cash per share (a 60% premium). The deal was already unanimously approved by both boards and is expected to close in Q4 2019. (Sep.)

Including Fibrocell's net debt, the transaction is valued at \$63.3m. Both companies have advanced candidates for multiple types of epidermolysis bullosa (EB), a rare genetic disease that leads to fragile skin prone to blistering, erosion, and peeling. The partners first collaborated in April 2019 when CCP in-licensed exclusive US development and commercialization rights to Fibrocell's lead potential gene therapy candidate FCX007, an autologous dermal fibroblast, which has US orphan, fast-track, rare pediatric, and regenerative medicine advanced therapy designations as well as orphan status in Europe. A Phase III trial in recessive dystrophic EB was initiated in July 2019 and a BLA filing is anticipated in 2021; with a 28% likelihood of approval (4% above average), according to Biomedtracker. Fibrocell originally gained US rights to FCX007, as well as the rest of its biologics pipeline, under a 2012 deal with **Intrexon**. Fibrocell is also evaluating FCX013 in a Phase I/II trial initiated in August 2018 for moderate to severe scleroderma (for which it has fast track and orphan status), and has additional research programs in arthritis and other related conditions. The combined company will focus on advancing Fibrocell's two clinical programs as well as CCP's own Phase IIb CCP020 (diacerein) for epidermolysis bullosa simplex (EBS) and other forms of EB. Investment Banks/Advisors: Canaccord Genuity Inc. (Fibrocell Science Inc.)

#### CURRAX PHARMACEUTICALS LLC NALPROPION PHARMACEUTICALS INC.

**Currax Pharmaceuticals LLC** acquired 2018 start-up **Nalpropion Pharmaceuticals Inc.**, gaining worldwide rights to Nalpropion's sole product, the FDA-approved *Contrave* (bupropion/naltrexone; sold in the EU as *Mysimba*) weight-loss medication. (Sep.)

Currax's predecessor company Pernix (acquired by Currax after it filed for bankruptcy in February 2019) owned a 10% stake in Nalpropion, formed by Pernix in April 2018 as a special purpose vehicle to acquire the assets (mainly *Contrave* (NB32)) of the now-defunct Orexigen Therapeutics, after Orexigen itself filed for Chapter 11 in March 2018. *Contrave* was approved in 2014 for adult patients with a body mass index between 27 or

greater (overweight) and 30 or greater (obese) who have at least one weight-related condition such as high blood pressure (hypertension), Type 2 diabetes, or high cholesterol (dyslipidemia). Currax also announced this month the acquisition of North American rights to **OptiNose's Onzetra Xsail** (sumatriptan nasal powder), an FDA-approved acute migraine treatment. In addition to *Contrave* and *Onzetra*, Currax now also has from Pernix's portfolio *Zohydro* (hydrocodone bitartrate) for pain (acquired from **Zogenix** in 2015); *Silenor* (doxepin hydrochloride) for insomnia (from its 2012 acquisition of Somaxon); and *Treximet* (naproxen sodium/sumatriptan) for migraine, gained from **GlaxoSmithKline PLC** under a 2014 deal.

#### GLAXOSMITHKLINE PLC SITARI PHARMACEUTICALS

**GlaxoSmithKline PLC** is acquiring **Sitari Pharmaceuticals** for an undisclosed sum. (Sep.)

In 2013, Avalon Ventures and GSK created a venture in which they sought to create and fund at least ten biopharma start-ups in the San Diego area to be incubated by COI Pharmaceuticals. (To date they established eight companies.) Under the agreement, Avalon identified and assessed early drug development candidates and granted GSK an option to acquire each company at the point of lead clinical candidate identification. Sitari was formed in late 2013 and was the first company born out of that collaboration. At the time Sitari received \$10m in Series A funding. The firm's intellectual property comes out of research from **Stanford University** surrounding the transglutaminase 2 (TG2) pathway. When exposed to gluten, the TG2 enzyme in the gut triggers an immune response that results in intestinal inflammation and disease pathogenesis. Sitari has been using the IP to develop TG2 inhibitors for celiac disease. The lead program is in preclinical studies. Currently, the only effective therapy for celiac is adherence to a strict gluten-free diet.

#### H. LUNDBECK AS ALDER BIOPHARMACEUTICALS INC.

Neuro-focused Danish pharmaco **H. Lundbeck AS** agreed to acquire public US biotech **Alder BioPharmaceuticals Inc.** for up to \$20 in cash per share. The transaction was already unanimously approved by Alder's board and is expected to close in Q4 2019. (Sep.)

Lundbeck is mainly interested in Alder's eptinezumab (ALD403), an intravenous monoclonal antibody (mAb) candidate for migraine prevention, that targets the calcitonin gene-related peptide (CGRP). Alder submitted a BLA to the FDA for eptinezumab in February 2019, with a PDUFA action date of February 21, 2020. It has a 91% likelihood of approval (8% above

average). Once approved, eptinezumab will be the first US-marketed IV CGRP therapy for migraine prevention. For the acquisition, Lundbeck will begin a tender offer of \$18 in cash per share (a 97% premium) initially (equal to \$1.5bn), plus one non-tradeable contingent value right (CVR) that entitles Alder to an additional \$2 per share (another \$167m) upon EMA approval of eptinezumab. Net of cash, the total transaction value is \$1.95bn. Lundbeck will fund the deal using both existing cash and additional financing. Lundbeck expects to submit an MAA for eptinezumab in the EU during 2020, followed by regulatory submissions globally, including China and Japan. The candidate is highly complementary to Lundbeck's neurology pipeline, enabling it to expand into migraine prevention. Alder also expects eptinezumab to have other indications, including acute migraine (for which a Phase III trial is planned in 2H 2019), to further back Lundbeck's future pipeline expansion. Alder also has a preclinical mAb for migraine prevention, ALD1910, designed to inhibit pituitary adenylate cyclase-activating polypeptide (PACAP). The current deal supports and accelerates Lundbeck's revenue growth strategy (announced in February 2019) in which the company recognized the need to strengthen its pipeline and portfolio with assets in all phases (and in those CNS diseases for which it doesn't currently have a presence) and improve its reach into Asian markets. Investment Banks/Advisors: MTS Health Partners; PJT Partners (H. Lundbeck AS); Centerview Partners LLC (Alder BioPharmaceuticals Inc.)

#### SWEDISH ORPHAN BIOVITRUM AB DOVA PHARMACEUTICALS INC.

**Swedish Orphan Biovitrum AB** (Sobi) signed a definitive agreement to acquire **Dova Pharmaceuticals Inc.**, a public three-year-old hematology drug company. The total potential value of the deal could hit \$915m, including \$27.50 per Dova share in cash (a 55% premium to the 10-day pre-announcement market average) and contingent value rights entitling Dova shareholders to \$1.50 per share. (Sep.)

Dova was formed by PBM Capital in 2016 to commercialize the thrombocytopenia therapy *Doptelet* (avatrombopag), a thrombopoietin receptor agonist that is approved in the US and EU for thrombocytopenia in adults with chronic liver disease and in the US for chronic immune thrombocytopenia (EU filing is expected next year). The treatment is also in Phase III for chemotherapy-induced thrombocytopenia; approval in this indication will trigger the CVR payments. Sobi's main business is in rare diseases. The acquisition of Dova will strengthen the firm's presence in the blood disorder market beyond hemophilia, and will also give it

an increased presence in the US. Dova brings about 125 staffers to Sobi through the deal, of which about 60% are specialists in hematology. Investment Banks/Advisors: Morgan Stanley & Co. (Swedish Orphan Biovitrum AB)

#### VERTEX PHARMACEUTICALS INC. SEMMA THERAPEUTICS INC.

**Vertex Pharmaceuticals Inc.** is paying \$950m in cash to acquire closely held diabetes-focused firm **Semma Therapeutics Inc.** (Sep.)

Post-transaction, Semma will operate as a Vertex subsidiary and its current president and CEO Bastiano Sanna, PhD, will continue in the president role. In addition, Semma's founder and SAB chair Douglas Melton, PhD, will keep his position and offer oversight and guidance on the R&D programs. Semma is focused on using encapsulated stem cell-derived human islets as a curative treatment for Type I diabetes. The company has found a way to produce large amounts of functional human pancreatic beta cells that can restore insulin secretion and ameliorate hypoglycemia. Its encapsulation technology offers a way to protect the cells from the immune system and allow for implantation of an islet-cell filled device without the need for patient immunosuppression. In June, Semma presented data demonstrating preclinical proof-of-concept of human stem cell-derived islets in primates and pigs. Semma's approach is the only islet cell transplantation program to have shown both positive c-peptide release--a marker of insulin secretion--and positive glycemic control of experimentally induced diabetes. Semma raised \$158m in its first two financing rounds from investors including Eight Roads Ventures, Cowen Healthcare Investments, MPM Capital, F-Prime Capital Partners, Arch Venture Partners, **Medtronic**, and 2015 collaboration partner **Novartis**. The acquisition represents Vertex's first move into the diabetes space as the firm is looking to expand beyond the area of cystic fibrosis. Just three months ago Vertex agreed to pay up to \$1bn to acquire **Exonics Therapeutics**, which is developing gene editing therapies for Duchenne muscular dystrophy.

#### ZHEJIANG HISUN PHARMACEUTICAL CO. LTD.

**Hisun BioRay Biopharmaceutical Co. Ltd.** Private equity firm PAG will pay \$540m to take a majority stake (58%) in **Hisun BioRay Biopharmaceutical Co.**, a division of **Zhejiang Hisun Pharmaceutical Co. Ltd.** (which retains a 42% ownership). (Sep.)

Hisun BioRay was formed earlier this year to develop, manufacture, and sell antibody therapeutics for cancer and autoimmune diseases. The company's first drug

on the market, *Anbainuo* (recombinant human tumor necrosis factor- $\alpha$  receptor II), is an etanercept biosimilar for autoimmune conditions including psoriasis, ankylosing spondylitis, and rheumatoid arthritis. Hisun BioRay has more than ten other projects in its pipeline, with the *Humira* biosimilar *Anjianning* due to hit the market in China near year-end. The investment by PAG (which came after Hisun entertained offers from 40 other interested parties) marks the largest ever for a Chinese biotech and provides Hisun BioRay with funds to accelerate R&D activities and expand the company's presence in the biosimilars and biologics markets.

#### ALLIANCES

##### 3B PHARMACEUTICALS GMBH CLOVIS ONCOLOGY INC.

**Clovis Oncology Inc.** licensed global rights (excluding Europe) to a peptide-targeted radionuclide therapy and an imaging agent targeting fibroblast activation protein alpha (FAP) from **3B Pharmaceuticals GmbH**. (Sep.)

Under terms of the deal, the partners will also discover and develop radiopharmaceuticals for three other undisclosed targets, to which Clovis will have worldwide rights. Clovis pays \$12m up front, milestones, and royalties ranging from the single to low-double digits. (*Strategic Transactions* estimates 1-29%.) Clovis is responsible for three 3BP FTEs in addition to external costs during the preclinical stage of the deal. FAP is highly expressed in cancer-associated fibroblasts found in epithelial cancers including breast, lung, colorectal, and pancreatic tumors. Using a FAP-targeted radiopharmaceutical agent results in the emission of ionizing radiation by cancer-associated fibroblasts surrounding the targeted area, resulting in DNA damage to tumor cells. A review of 3B's pipeline notes that the companies have named two preclinical solid tumor projects under their deal, 3B201 and 3B202. Clovis moves into the radiopharmaceuticals arena via the deal with 3B. The collaboration comes at a time when the company is seemingly struggling to turn strong profits for its PARP inhibitor *Rubraca* (rucaparib), which was approved by the FDA in 2016 and launched with stiff competition from **AstraZeneca's** PARP inhibitor *Lynparza* (olaparib). While market analysts and investors reacted negatively to Clovis' decision to pay \$12m up front for a preclinical asset under the current deal, the collaboration could provide an opportunity for the company to further diversify its offerings.

##### ABBVIE INC. IDERA PHARMACEUTICALS INC.

**Idera Pharmaceuticals Inc.** and **AbbVie Inc.** forged a trial collaboration to study

combinations of ABBV368, tilsotolimod, nab-paclitaxel, and/or ABBV181. (Sep.)

The parties seek to determine whether or not the combinations can stimulate the immune system and produce anti-tumor responses. The collaboration will study three treatment arms: AbbVie's Phase I OX40 agonist ABBV368 plus Idera's Phase I TLR-9 agonist tilsotolimod; ABBV368 plus tilsotolimod and the chemotherapy nab-paclitaxel; and ABBV368 plus tilsotolimod, nab-paclitaxel, and AbbVie's Phase I programmed cell death 1 (PD-1) antagonist ABBV181. The planned Phase Ib trial will determine the safety, tolerability, pharmacokinetics, and preliminary efficacy of combinations of ABBV368 plus tilsotolimod in patients with recurrent or metastatic head and neck squamous cell carcinoma. Idera will supply tilsotolimod and AbbVie will conduct the study.

#### **ALEXION PHARMACEUTICALS INC. BRIDGEBIO PHARMA INC.**

*Eidos Therapeutics Inc.*

**Eidos Therapeutics Inc.** licensed **Alexion Pharmaceuticals Inc.** exclusive rights to develop and commercialize AG10 in Japan. (Sep.)

Eidos gets \$25m up front, an equity investment of \$25m, plus milestones and royalties. AG10 is designed to bind and stabilize transthyretin (TTR) protein in the blood to treat transthyretin amyloidosis (ATTR). The compound is currently in Phase III in the US and Europe for ATTR cardiomyopathy and is expected to enter Phase III for ATTR polyneuropathy by the end of 2019.

#### **AMERIGEN PHARMACEUTICALS INC. INVENTIA HEALTHCARE LTD.**

**Amerigen Pharmaceuticals Inc.** licensed exclusive US sales, marketing, and distribution rights to India generics company **Inventia Healthcare Ltd.**'s paliperidone extended-release (ER) tablets and tolterodine ER capsules. (Sep.)

Inventia retains manufacturing rights and will supply the products. It has an Indian manufacturing facility to formulate approved oral products across a range of delivery dosage forms. A generic equivalent to **Janssen's Invega**, the paliperidone ANDA was approved in the US in June 2019. The 5-HT<sub>2</sub> and partial dopamine D<sub>2</sub> antagonist is an antipsychotic medicine for schizophrenia. The ANDA for tolterodine, a generic to **Pfizer's Detrol LA** for overactive bladder, was approved just last month. Tolterodine is commercialized in India by **Dr. Reddy**. The current deal enables Inventia to increase its global reach, while allowing Amerigen to expand its portfolio, which is mainly focused on externally sourced orally formulated APIs that are challenging to develop, require specialized technologies to manufacture, or have

other regulatory and intellectual property difficulties in gaining US and Chinese approval. Earlier this year, the FDA approved the company's ANDAs for penicillamine capsules (a generic equivalent to **Bausch Heath's Cuprimine** for Wilson's disease, cystinuria, and rheumatoid arthritis) and ER capsules of mixed amphetamine salts (a generic version of **Shire's Adderall XR** for ADHD).

#### **AMPLYX PHARMACEUTICALS INC. NOVARTIS AG**

**Amlyx Pharmaceuticals Inc.** licensed exclusive worldwide rights to **Novartis AG's** monoclonal antibody, MAU868, for treatment and prevention of BK virus (BKV)-related disease. (Sep.)

Novartis was assessing safety, tolerability, and efficacy of the compound in preclinical studies for the prevention of BKV infection in kidney transplant recipients before suspending development for unknown reasons late last year. A polyomavirus, initial BK infection is asymptomatic, but it can remain dormant in the kidney or bladder; if reactivated by a weakened immune system, serious disease may result. MAU868 targets VP1, the major viral capsid protein of BKV, necessary for the virus to bind to and infect new cells. Amlyx believes MAU868 will protect against BKV reactivation in kidney transplant and hematopoietic stem cell transplant patients, thus preventing, respectively, renal allograft failure and hemorrhagic cystitis (an inflammatory bladder disease). Amlyx anticipates initiating two Phase II proof-of-concept studies of the mAb by year end.

#### **ATOMWISE INC. JIANGSU HANSOH PHARMACEUTICAL GROUP CO. LTD.**

AI-focused drug discovery start-up **Atomwise Inc.** and Chinese biotech **Jiangsu Hansoh Pharmaceutical Group Co. Ltd.** (Hansoh) agreed to collaborate on the design and discovery of potential drug candidates for up to 11 undisclosed target proteins across numerous therapeutic areas, including oncology. (Sep.)

Researchers from both companies will partner their respective capabilities with an aim to improve the chances for success and decrease discovery and development timelines. Atomwise will perform direct hit discovery, hit-to-lead selection, and lead optimization. Hansoh contributes its biological assay and medicinal chemistry expertise and will be responsible for leading preclinical and clinical development activities for potential compounds to which it receives worldwide development and commercialization rights in all fields. Atomwise will receive undisclosed technology access and option exercise payments, royalties, and potential fees from future sublicensing or sale of assets.

The total potential value of the deal if all projects succeed is expected to reach blockbuster potential, according to the companies. Atomwise's *AtomNet* structure-based drug design platform, based on deep convolutional neural networks, can analyze a chemical space of billions and billions of compounds to identify a small subset with high specificity. The technology's statistical approach extracts insights from millions of experimental affinity measurements and thousands of protein structures to predict the binding of small molecules to proteins. Although no specific therapy areas other than cancer were disclosed, Hansoh's areas of focus include oncology, anti-infectives, diabetes, and CNS, cardiovascular, and gastrointestinal disorders.

#### **BIOMOTIV LLC BRISTOL-MYERS SQUIBB CO.**

**Bristol-Myers Squibb Co.** agreed to become a limited partner in **BioMotiv LLC**. Together they will establish start-ups, which BMS might eventually buy. (Sep.)

BioMotiv was founded in 2012 around the business model of in-licensing assets from academic institutions, developing programs through proof-of-concept studies, and then out-licensing the drugs or technologies to a partner. It has done such deals with **Takeda** and **Biogen**. Now, BioMotiv has given BMS the option to fund selected projects (targeting great unmet need), around which the partners would form and fund new companies. Once a preclinical candidate is identified, BMS would have the option to acquire that company under pre-agreed terms. Therapeutic areas were not disclosed, although BioMotiv's previous alliances with Takeda and Biogen involved immunology, inflammation, and cardio-metabolic and neurological diseases. BMS is currently most active in oncology.

#### **BOEHRINGER INGELHEIM INTERNATIONAL GMBH INFLAMMASOME THERAPEUTICS INC.**

**Boehringer Ingelheim International GmbH** and **Inflammasome Therapeutics Inc.** agreed to co-develop up to three candidates for retinal diseases. (Sep.)

BI will provide up to \$160m in up-front, R&D, and milestone payments, plus tiered royalties and additional commercialization milestones. The deal combines compounds from the Big Pharma's retinal disease pipeline with Inflammasome's intravitreal (IVT) drug delivery technologies. Inflammasome's long-acting degradable IVT implant enables the administration of compounds to the eye as sustained-release depot formulations. Although specific candidates weren't disclosed, BI has compounds for retinal diseases in various phases of development, including Phase II BI1467335 for diabetic retinopathy,

Phase I BI754132 for age related macular degeneration (AMD), and BI836880, in Phase I for wet AMD.

**BOEHRINGER INGELHEIM  
INTERNATIONAL GMBH  
LUPIN LTD.**

**Boehringer Ingelheim GmbH** licensed exclusive worldwide development and commercialization rights to **Lupin Ltd.**'s LNP3794 mitogen-activated ERK kinase (MEK) inhibitor compound for difficult-to-treat cancers. (Sep.)

In exchange, Lupin gets \$20m up front, specified clinical, regulatory, and sales milestones of more than \$700m, plus double-digit royalties. LNP3794 has demonstrated preclinical activity as a single agent as well as in combination with other therapies. MEK inhibitors and BI's own K-Ras inhibitors are known to have complementary mechanisms of action. Ras is the most frequently mutated oncogene known in cancer, with K-Ras being the most common subtype occurring in cancers of the pancreas, colon, biliary tract, and lung. BI plans to develop LNP3794 in combination with one compound from its pipeline to treat K-Ras-dependent gastrointestinal and lung cancers. BI's preclinical research has already shown that its own K-Ras inhibitors in combination with MEK inhibitors resulted in increased anti-tumor activity. Based on partnerships with both **Vanderbilt University** and **MD Anderson**, BI's cancer cell-directed research compounds include potential therapies to restrain uncontrolled receptors and intracellular signaling, correct abnormal pathways, re-establish apoptosis, and restore transcriptional control.

**BOEHRINGER INGELHEIM  
INTERNATIONAL GMBH  
ONCOHEROES BIOSCIENCES INC.**

**Boehringer Ingelheim International GmbH** licensed **Oncoheroes Biosciences Inc.** exclusive worldwide R&D and commercialization rights (and related IP) to its volasertib (BI6727), a polo-like-kinase 1 (PLK1) inhibitor for cancer. (Sep.)

The Big Pharma was advancing the compound in several cancer indications, but suspended development following a 2016 Phase III study in adult acute myeloid leukemia patients that failed to meet its primary endpoint. Oncoheroes plans to further develop and eventually sell volasertib for rhabdomyosarcoma (RMS), the most common pediatric soft tissue sarcoma, and other childhood cancer indications. BI's additional studies of volasertib (all since suspended) included a Phase I European study of the compound as a single agent in children with leukemia or refractory solid tumors. A recommended Phase II dose for children has been defined. Because of preclinical evidence demonstrating

volasertib's ability to reduce the activity and stability of the fusion proteins thought to be responsible for many cases of the disease, it might prove an effective therapeutic agent against RMS. Clinical studies are expected to begin in 2020. The pediatric oncology-focused 2017 start-up has an internal discovery unit, but is also looking to in-license development candidates that have previously shown some clinical promise. The company's 2HIT is a discovery-stage program for medulloblastoma, a malignant CNS tumor. A preclinical candidate for 2HIT is expected in late 2020.

**CIDARA THERAPEUTICS INC.  
MUNDIPHARMA INTERNATIONAL  
CORP. LTD.**

**Cidara Therapeutics Inc.** licensed **Mundipharma International Corp. Ltd.** exclusive global rights outside the US and Japan to develop and commercialize intravenous rezafungin for treating invasive fungal infections. (Sep.)

For the rights, Mundipharma will pay Cidara \$30m up front and make a \$9m equity investment in the firm. Cidara is also eligible for \$42.4m in development funding to support the global Phase III ReSTORE and ReSPECT trials, up to \$534.4m in development, regulatory, and commercial milestones, plus sales royalties in the teens. In addition to IV rezafungin, Cidara also granted Mundipharma an option to obtain exclusive licenses to develop, register, and commercialize rezafungin in subcutaneous and other formulation for administration. Mundipharma also has a co-exclusive worldwide license to manufacture the drug. Cidara will continue to lead the Phase III development programs with the support from Mundipharma. The parties may choose to pursue additional indications or formulations of rezafungin. Once-weekly rezafungin is a broad-spectrum, long-lasting echinocandin antifungal in development as a first-line treatment of candidemia and invasive candidiasis, including prophylaxis of invasive fungal infections in patients undergoing allogeneic blood and marrow transplantation. For the latter indication, there has not been an approved therapy for more than 13 years. Current treatments for systemic fungal infections include polyenes, azoles, and echinocandins, all of which have significant limitations such as toxicities, drug-drug interactions, low or variable exposure, and increasing resistance. Cidara recently reported positive topline results from Part B of the global Phase II STRIVE trial of rezafungin. Cidara chose to partner with Mundipharma because of its commercial presence in more than 120 markets in addition to annual sales of over \$2.2bn.

**COPERNICUS THERAPEUTICS INC.  
CAN-FITE BIOPHARMA LTD.**

*Wize Pharma Inc.*

**Copernicus Therapeutics Inc.** licensed **Wize Pharma Inc.** exclusive global rights to develop, manufacture, and commercialize non-viral gene therapies for chorioideremia (CHM) based on Copernicus' technology. Wize also has the right to sublicense. (Sep.)

Wize will pay an undisclosed up-front fee, development milestones (in cash or stock), and high-single or low-double-digit sales royalties (*Strategic Transactions* estimates 7-29%). Wize will also pay Copernicus fees to fund and execute the development plan leading to the completion of the Phase I/II clinical trial. Copernicus's technology enables the development of effective non-viral gene therapies for ophthalmic indications without toxicity. CHM is a rare, degenerative, inherited retinal disorder that mostly affects males and leads to blindness. There are no FDA-approved treatments for the condition. Based on Copernicus IP, Wize is developing WP-REP1, consisting of a DNA-compacted nanoparticle (NP) that is administered via intraocular injection. The therapy is designed to provide a functioning CHM gene to photoreceptors and retinal pigment epithelial (RPE) cells. WP-REP1 has demonstrated proof-of-concept in preclinical studies. The companies may choose to expand the collaboration to other ophthalmic indications based on the Copernicus technology.

**CURRAX PHARMACEUTICALS LLC  
OPTINOSE INC.**

**Currax Pharmaceuticals LLC** (formerly Pernix Therapeutics) gained exclusive US, Canadian, and Mexican marketing rights to **OptiNose Inc.**'s *Onzetra Xsail* (sumatriptan) dry powder formulation acute migraine treatment. (Sep.)

Currax will provide \$4.48m up front (\$750k of which will be held in escrow), a \$1m regulatory milestone, plus a one-time 10% royalty on net sales in excess of \$3m during calendar year 2020. FDA-approved in 2016, *Onzetra Xsail* is administered via OptiNose's breath-powered powder exhalation delivery system (EDS), which consists of a reusable device with a flexible and adjustable fit mouthpiece with an assembly to pierce the medication capsule. Currax also has a license to certain patent rights to the EDS. Pernix--Currax's predecessor company, which filed for bankruptcy in February 2019 and was acquired by Currax after that--has US rights to another marketed migraine therapy, *Treximet* (naproxen sodium/sumatriptan), which it gained from **GlaxoSmithKline PLC** under a 2014 deal.

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**DAIICHI SANKYO CO. LTD.  
MITSUBISHI CHEMICAL  
HOLDINGS CORP.**

*Mitsubishi Tanabe Pharma Corp.*

**Mitsubishi Tanabe Pharma Corp.** granted **Daiichi Sankyo Co. Ltd.** commercialization rights in Brazil for an intravenous infusion formulation of edaravone for amyotrophic lateral sclerosis (ALS). (Sep.)

Edaravone (MCI186) was first approved for ALS as *Radicut* in Japan in June 2015, followed by South Korea (12/15), the US (5/17) and Canada (10/18)--it's marketed in the latter two territories as *Radica-*-Switzerland (1/19), and most recently, China (8/19). The drug is a free radical scavenger that protects motor neurons from oxidative stress to delay ALS disease progression. Daiichi also has rights of first negotiations for commercialization of the IV dosage form in Central and South American countries other than Brazil as well as for other dosage forms of edaravone in countries in Central and South America, including Brazil. Daiichi is responsible for filing a regulatory application and will commercialize the product in Brazil upon its approval.

**EVOTEC SE  
TAKEDA PHARMACEUTICAL CO. LTD.**

**Evotec SE** and **Takeda Pharmaceutical Co. Ltd.** entered into a multi-year agreement for the discovery and development of new therapies across a variety of indications. (Sep.)

Under terms of the deal, Evotec will use its drug discovery technologies to uncover up to five programs in the areas of oncology, gastroenterology, neuroscience, and rare diseases. Takeda has options at lead series and upon delivery of preclinical candidates to license development and commercialization rights to any candidates. Takeda made an undisclosed up-front payment and could hand over up to \$170m per program in total milestones (\$850m for all five) plus tiered royalties. Takeda and Evotec have worked together in the past. In 2003, **Evotec Neurosciences** and Takeda penned a four-year collaboration to discover new treatments for Alzheimer's disease.

**FLEXION THERAPEUTICS INC.  
XENON PHARMACEUTICALS INC.**

**Flexion Therapeutics Inc.** gained exclusive worldwide development and commercialization rights to **Xenon Pharmaceuticals Inc.**'s XEN402, a Nav1.7 sodium channel inhibitor for the non-opioid management of post-operative pain. The license includes associated patents and related non-clinical, clinical, and manufacturing assets. (Sep.)

Xenon gets \$3m up front; up to \$9m in manufacturing, development, and regu-

latory milestones (through the initiation of a Phase II proof of concept trial); up to \$40.75m in development and regulatory milestones (following Phase II PoC); up to \$75m in commercialization milestones; plus future sales royalties ranging from the mid-single to low double-digits (*Strategic Transactions* estimates 4-29%). Flexion will also assume Xenon's remaining obligation under a 2012 agreement (since terminated) to pay a low single-digit percentage sales royalty to **Teva Pharmaceuticals**. XEN402 was previously tested in multiple human clinical trials, which demonstrated good efficacy when delivered to the target site at high concentration. The candidate had reached Phase II trials before it was suspended after failing to meet primary and secondary endpoints in osteoarthritis (OA), post-herpetic neuralgia, and neuropathic pain indications. Flexion plans to conduct a preclinical program called FX301, in which it will formulate XEN402 as an extended-release hydrogel. Flexion's thermosensitive formulation technology aims to transition the drug compound from a liquid to a gel within minutes after injection to provide local delivery near target nerves for up to a week. Initially focusing its administration as a peripheral nerve block to control post-operative pain, FX301 is expected to enable ambulation and early rehabilitation following musculoskeletal surgery, unlike local anesthetics that may inhibit motor function. Flexion anticipates initiating clinical trials for FX301 in 2021. Flexion's *Zilretta* (triamcinolone acetonide) is its injectable extended-release corticosteroid suspension to manage OA knee pain that was launched in 2017. It's also developing FX201--a preclinical interleukin-1 receptor antagonist in-licensed from **GeneQuine Biotherapeutics**--for OA pain; the locally administered gene therapy is expected to enter first-in-human clinical trials sometime this year.

**GLAXOSMITHKLINE PLC  
VBI VACCINES INC.**

**VBI Vaccines Inc.** and **GlaxoSmithKline PLC** are teaming up in a trial collaboration to study the combination of VBI1901 cancer immunotherapy with GSK's AS01B adjuvant system. (Sep.)

VBI1901 incorporates VBI's enveloped virus-like particle (eVLP) technology and has demonstrated potency in the Phase I/IIa trial for treating recurrent glioblastoma (GBM). VBI1901 is administered intradermally when adjuvanted with granulocyte-macrophage colony-stimulating factor. AS01B has shown its ability to boost T-cell mediated immunity. This is GSK's first partnership with a biopharma company to evaluate AS01B in the clinic and the first time it will be studied in oncology for GBM patients. AS01B has proven effective in

combination with the gE antigen in GSK's shingles vaccine *Shingrix*.

**GRUNENTHAL GMBH  
MESOBLAST LTD.**

**Grunenthal GmbH** licensed exclusive European and Latin American development and commercialization rights to **Mesoblast Ltd.**'s MPCo6ID (rexlemestrocil-L), an injectable cell therapy for chronic low back pain (CLBP) due to degenerative disc disease. (Sep.)

MPCo6ID uses allogenic mesenchymal precursor cells (MPCs), which secrete multiple factors that stimulate new proteoglycan and collagen synthesis by chondrocytes. MPCs have also been shown to produce anti-inflammation factors. In a US Phase II trial (completed in 2013), MPCo6ID demonstrated that a single intra-discal injection resulted in significant improvements in CLBP intensity, functionality, and disc stability for at least three years. The candidate is currently undergoing a US Phase III trial with top-line results expected in 2020. MPCo6ID has a 54% likelihood of approval (2% above average). Both companies will collaborate on the study design for a European confirmatory Phase III trial. Mesoblast will get \$15m up front, up to \$1bn in total milestones (includes \$135m in precommercialization milestones, including \$20m upon approval to begin the confirmatory Phase III trial in Europe and \$10m for certain clinical and manufacturing outcomes), plus tiered double-digit sales royalties. The deal advances the approval pathway for one of Mesoblast's lead candidates by a European partner with a known expertise in the pain management field and a focus on the underlying pathophysiology of the disease rather than just addressing the symptoms. Grunenthal currently sells multiple pain medications--including *Nucynta ER* (tapentadol) for CLBP, cancer pain, and diabetic peripheral neuropathy--and has five NCE projects in Phase II or III as well as multiple pain programs in early development.

**IFM THERAPEUTICS LLC  
IFM Due**

**NOVARTIS AG**

**IFM Therapeutics LLC**, through its **IFM Due** division, agreed to collaborate with **Novartis AG** on the development of cGAS/STING (cyclic GMP-AMP synthase/stimulation of interferon genes) pathway inhibitors to treat various inflammatory and autoimmune diseases. (Sep.)

Through fixed payments, Novartis will fully fund IFM Due's R&D costs for the cGAS/STING program in exchange for the company to acquire the subsidiary company outright. If Novartis exercises that purchase option, it will pay a total of up to \$840m (including an up-front



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option closing fee and other contingent consideration). Within the innate immune system, the cGAS/STING pathway functions to sense cytosolic DNA (a signal of cellular danger) and thus triggers an inflammatory response. Mutations in the activation of this pathway can lead to excessive production of interferon and other pro-inflammatory cytokines that can cause a range of rare diseases, including Aicardi-Goutieres syndrome (AGS), STING-associated vasculopathy with onset in infancy, and systemic lupus erythematosus, as well as more common conditions such as nonalcoholic steatohepatitis, chronic obstructive pulmonary disease, age-related macular degeneration, and Parkinson's disease. Founded in February 2019, IFM Due houses its parent company's two small-molecule preclinical pipeline programs: STING antagonists to prevent the stimulation that leads to an excessive immune response (expected to enter the clinic in 2021), and cGAS inhibitors, which aim to block the pathway at a more upstream node. Back in April 2019, Novartis paid \$1.58bn to acquire one of IFM's other subsidiaries, **IFM Tre**, which is focused on inhibition of the NLRP3 inflammasome, a multi-protein intracellular innate immune signaling receptor.

#### INTELLIPHARMACEUTICS INTERNATIONAL INC. TRIS PHARMA INC.

Just a month after their first agreement, **Tris Pharma Inc.** licensed exclusive US marketing, sales, and distribution rights to a second CNS generic from **Intellipharmaceutics International Inc.**; this time gaining the latter's depression drug desvenlafaxine succinate extended-release (ER) tablets in the 50mg and 100mg strengths. The partners also entered a concurrent commercial supply agreement. (Sep.)

Desvenlafaxine succinate received tentative ANDA approval from the FDA for major depressive disorder in February 2019. The dual serotonin and norepinephrine reuptake inhibitor is the generic equivalent to **Pfizer's Pristiq**. Back in August, Tris licensed exclusive US rights to **Intellipharmaceutics'** quetiapine fumarate ER tablets, a generic equivalent to **AstraZeneca's Seroquel XR** schizophrenia drug. Under 2016 deal, **Intellipharmaceutics** had a long-term profit-sharing arrangement and US licensing arrangement with **Mallinckrodt** involving desvenlafaxine (as well as quetiapine fumarate and lamotrigine (a generic to **GlaxoSmithKline's Lamictal XR** for epilepsy), but that agreement was terminated in April 2019.

#### JW PHARMACEUTICAL CORP. SIMCERE PHARMACEUTICAL GROUP

**JW Pharmaceutical Corp.** licensed **Simcere Pharmaceutical Group** exclusive rights to develop and commercialize its Phase IIb gout candidate URC102 in China, Hong Kong, and Macau. (Sep.)

JW will receive an undisclosed up-front payment, milestones, and sales royalties. **Simcere** will conduct clinical trials and handle registration and commercialization of URC102 in the licensed territory. URC102 is a small molecule inhibitor of the urate transporter protein (URAT-1) designed to reduce the serum uric acid levels in gout patients.

#### KEMPHARM INC.

**KemPharm Inc.** granted an affiliate of VC firm Gurnet Point Capital (GPC) exclusive worldwide rights to its KP415 and KP484 prodrug candidates for attention deficit/hyperactivity disorder (ADHD). (Sep.)

With an NDA submission expected by the end of this year, Phase III KP415 is an oral, thin film prodrug of methylphenidate (MPH) generated using **KemPharm's LAT (Ligand Activated Therapy)** prodrug technology. Preclinical KP484 is a super-extended release prodrug of d-threo-MPH. **KemPharm** receives \$10m up front, up to \$63m in regulatory milestones (both prior to and upon approval), US sales milestone up to \$420m, plus tiered royalty payments on a product-by-product basis ranging from the high-single-digits up to a mid-twenties percentage for US sales, and in the low-to mid-single-digits in each country outside the US. GPC also has the option to exclusively license any other candidate developed by **KemPharm** containing serdexmethylphenidate (a prodrug of d-MPH) to treat ADHD or any other CNS disorder, including preclinical KP879 (for stimulant use disorder) and KP922 (for ADHD and Tourette's syndrome). **KemPharm** will manage all development activities, with GPC reimbursing the company for all development, regulatory, and commercialization expenses. A joint steering committee will be established to monitor development progress of the KP415 and KP484 programs. GPC will be responsible for all commercialization and manufacturing activities. **KemPharm** plans to apply the money to a concurrently announced amendment to its 2015 senior debt facility with Deerfield Management, potentially reducing its outstanding borrowings by up to \$30m in the aggregate. Investment Banks/Advisors: RBC Capital Markets

#### NEUCYTE INC. TRILLIUM THERAPEUTICS INC.

**NeuCyte Inc.** licensed exclusive worldwide development and commercialization rights to **Trillium Therapeutics Inc.**'s un-

disclosed refractory epilepsy compound for Dravet syndrome and related disorders. (Sep.)

In preclinical studies conducted by the **National Institutes of Health's National Institute of Neurological Disorders and Stroke (NINDS)**, the compound demonstrated safety and efficacy in both anti-seizure effectiveness over benchmark anti-epileptic drugs in eleven animal models and in NIH animal studies for drug-resistant epilepsy. **NeuCyte's SynFire** cell-based translational technology generates induced pluripotent stem cell (iPSC)-derived neural cells that exhibit the main characteristics of human neurons for target identification and validation, efficacy testing, in vitro disease modeling, neurotoxicity assessment, and disease modeling. When applying this platform to the **Trillium** compound, favorable efficacy and safety profiles were seen in **NeuCyte's** iPSC-derived models. The partners believe the anti-seizure compound, complementary to **NeuCyte's** existing CNS disease pipeline, could also potentially have other indications.

#### POLPHARMA SA Polpharma Biologics NOVARTIS AG

*Sandoz International GMBH*

**Polpharma Biologics** granted **Sandoz International GmbH** worldwide commercialization rights to its natalizumab biosimilar. (Sep.)

**Polpharma** is advancing the third-wave biosimilar under the name PBoo6 with its service partner **Bioeq GmbH** (a 2014 JV created with **Strungmann Group** to handle **Polpharma's** biosimilar pipeline development and regulatory activities). The humanized mAb, a biosimilar to **Biogen's Tysabri** (which was approved in 2004), is in Phase III for relapsing-remitting multiple sclerosis, with a market launch anticipated in 2021. Under the current deal, **Polpharma** will continue its development and manufacturing activities and supply **Sandoz** with the biosimilar, which it will distribute globally after its approval. Over the past year, **Sandoz** has supplemented its pipeline by in-licensing several other biosimilars, including recent deals with **Gan & Lee** (insulin) and **EirGenix** (trastuzumab).

#### PT KALBE FARMA TBK PT Kalbe-Genexine Biologics SHANGHAI HENLIUS BIOTECH INC.

**Shanghai Henlius Biotech Inc.** granted **PT Kalbe-Genexine Biologics** (KG Bio) exclusive rights to develop and sell the anticancer immunotherapy HLX10 in the Asia Pacific territory including the Philippines, Indonesia, Malaysia, Singapore, Thailand, Laos, Myanmar, Cambodia, Brunei, and Vietnam. (Sep.)

KG paid \$10m up front and is responsible

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for \$10m in R&D money to fund trials of two combination therapies that will be run by Henlius; \$22m in regulatory milestones; and \$650m in payments for sales achievements. HLX10 is a recombinant humanized anti-PD-1 monoclonal antibody injection in Phase III trials for esophageal and lung cancers; it is also in earlier trials for liver, squamous cell, and head and neck cancers. Henlius looks forward to the expanded Southeast Asian exposure it will gain through the collaboration. KG Bio is a joint venture formed in 2015 between **PT Kalbe Farma TBK** and **Genexine** to develop and sell biologics and mAbs in the ASEAN, Asia Pacific, and MENA regions.

#### **ROIVANT SCIENCES INC.**

*Altavant Sciences Inc.*

#### **ROIVANT SCIENCES INC.**

*Enzyvant Sciences Ltd.*

#### **ROIVANT SCIENCES INC.**

*Myovant Sciences Ltd.*

#### **SUMITOMO CHEMICAL CO.**

*Sumitomo Dainippon Pharma Co. Ltd.*

#### **ROIVANT SCIENCES INC.**

*Urovant Sciences Ltd.*

Through a non-binding memorandum of understanding (MOU), **Sumitomo Dainippon Pharma Co. Ltd.** (SDP) and **Roivant Sciences Inc.** plan to forge an alliance involving the clinical-stage assets of multiple Roivant biopharmaceutical divisions (known as vants). The potential partners will conduct due diligence and expect to sign a definitive agreement by the end of October 2019. (Sep.)

Roivant's business model is to create separate vant subsidiaries (it has 16 so far), each housing development pipelines within a specific disease focus. Under the proposed arrangement, SDP plans to assume Roivant's ownership interests in five of the vants--including **Altavant Sciences Inc.** (rare respiratory diseases), **Enzyvant Sciences Ltd.** (rare immunodeficiency diseases), **Myovant Sciences Ltd.** (women's health and prostate cancer), **Urovant Sciences Ltd.** (urinary disorders), and one undisclosed vant--and will have the option to acquire up to six additional biopharma vants. SDP would also gain access to Roivant's technology platforms, including **DrugOme** (accelerates pipeline acquisition and clinical development) and **Digital Innovation** (uses technology to improve business processes). In addition, SDP expects to enter contract agreements with Roivant's services vants **Alyvant** (big data-enabled technologies to improve sales and marketing effectiveness) and **Datavant** (protects, links, and connects external patient health data). The planned deal, which could involve up to 11 biopharmaceutical vants and more than 25 clinical programs (with product launches expected

between 2020 to 2022) is worth \$3bn up front, which SDP will pay in cash to Roivant upon the closing of the transaction. Altavant's oral tryptophan hydroxylase inhibitor RVT1201 (rodatristat ethyl) is in a Phase II trial initiated earlier this year for pulmonary arterial hypertension; top line results are expected in Q2 2020. Enzyvant's lead program is Phase III (BLA filed) RVT802, a regenerative therapy for the rare pediatric immune disease congenital athymia; a regulatory decision is expected in December 2019. Myovant completed Phase III trials for its oral GnRH receptor antagonist relugolix for uterine fibroids (and is on-track to submit a US NDA filing by the end of fiscal 2019); it also anticipates top-line results from Phase III studies in prostate cancer (by year end) and endometriosis (between Q1 and Q2 2020). Urovant's most advanced candidate is Phase III oral beta-3 adrenergic receptor agonist vibegron for overactive bladder (OAB); it plans to submit an NDA to the (FDA) by early 2020. If executed, the deal could give SDP's pipeline a necessary expansion. SDP has met with recent clinical disappointments--including napabucasin (which failed a Phase III study in pancreatic cancer in July) and SB623, a cell therapy partnered with **SanBio**, which didn't reach its primary endpoint in a Phase II ischemic stroke trial back in January. The Japanese firm also faces the upcoming loss (in early 2023) of exclusivity in the US for its top product **Latuda** (lurasidone), an antipsychotic with 2018 North American sales of \$1.66bn.

## FINANCINGS

### **ACADIA PHARMACEUTICALS INC.**

**Acadia Pharmaceuticals Inc.** (psychiatric drug development) netted \$271.7m through the public offering of 7.2m shares (including the overallotment) at \$40. The company will use some of the proceeds to fund the potential commercialization of lead candidate **Nuplazid** (pimavanserin) for dementia-related psychosis, for which it has a 60% likelihood of approval (8% above average), according to Biomedtracker; an NDA submission is expected next year. The small-molecule serotonin 5-hydroxytryptamine 2A (5-HT<sub>2A</sub>) receptor antagonist pimavanserin was initially launched in the US in April 2016 for hallucinations and delusions associated with Parkinson's disease psychosis. In addition to dementia-related psychosis, **Nuplazid** is also in Phase III for major depressive disorder (a 52% LOA, 1% above average) and in Phase II for schizophrenia (a Phase III trial in July 2019 failed to reach the primary endpoint). (Sep.)

Investment Banks/Advisors: Bank of America Merrill Lynch; Canaccord Genu-

ity Inc.; Cantor Fitzgerald & Co.; Cowen & Co. LLC; JMP Securities LLC; JP Morgan & Co.; Needham & Co. Inc.; SVB Leerink

### **AERIE PHARMACEUTICALS INC.**

Ophthalmic-focused **Aerie Pharmaceuticals Inc.** announced an upsized private placement of \$275m (net \$266.1m) aggregate principal amount of its 1.50% convertible senior notes due 2024 to qualified institutional buyers. The notes bear interest at a rate of 1.50% per annum payable semi-annually in arrears and can be converted in cash, stock, or a combination. The conversion rate will initially be 40.0400 shares of common stock per \$1k principal amount of notes, or \$24.98 per share (its shares are currently averaging \$22.44). The company also granted the initial purchasers a 13-day option to buy another \$41.25m notes. Aerie announced it is entering into capped call transactions and will use some of the proceeds to fund those transactions. Additional funds will be used to pay termination fees related to an existing senior secured credit facility, to fund clinical development, regulatory activities, and commercialization of its **Rhopressa** (netarsudil 0.02%) and **Rocklatan** (netarsudil 0.02%/latanoprost 0.005%) ophthalmic solutions, both for glaucoma/ocular hypertension, and for pipeline development and manufacturing. (Sep.)

### **ARTARA THERAPEUTICS INC.**

### **PROTEON THERAPEUTICS INC.**

Publicly traded **Proteon Therapeutics Inc.** and closely held **ArTara Therapeutics Inc.** are reverse merging via an all-stock transaction. Concurrently, a syndicate of healthcare investors have agreed to invest \$42.5m in the combined company when the transaction closes. The financing will fund development of ArTara's Phase III TARA002 for lymphangiomas and Phase IIa IV choline chloride for intestinal failure associated liver disease. (Sep.)

### **BELLUS HEALTH INC.**

**Bellus Health Inc.** netted \$73.8m in its initial public offering on the Nasdaq of 11.18 million common shares (including partial exercise of the overallotment) at \$7.10. (Sep.)

Investment Banks/Advisors: Bloom Burton & Co.; Cowen & Co. LLC; Guggenheim Partners LLC; Jefferies & Co. Inc.; Robert W. Baird & Co. Inc.

### **BIONTECH SE**

**BioNTech SE** (immuno-oncology) has filed for its initial public offering on the Nasdaq Global Market. The company intends to sell 13.2 million American Depositary Shares at a price range between \$18-20 per ADS. (Sep.)

Investment Banks/Advisors: Bank of America Merrill Lynch; Bryan Garnier & Co.; Canaccord Genuity Inc.; JP Morgan Chase & Co.; Kempen & Co.; Mirae Asset Financial Group; SVB Leerink; UBS Investment Bank

#### CELYAD SA

**Celyad SA** (CAR-T cell therapies for cancer) netted \$18.8m through a global offering. In the US, the company sold 1.7 million American Depositary Shares (equivalent to 1.7 million common) at \$10 apiece, while 282,609 ordinary shares were issued in Europe and certain other countries outside of the US and Canada. Money is earmarked for continued development of pipeline projects CYAD01 (Phase I autologous CART for acute myeloid leukemia, myelodysplastic syndrome, metastatic colorectal cancer (mCRC), and other solid/blood cancers), CYAD101 (Phase I allogeneic therapy for mCRC), and additional candidates. (Sep.)

Investment Banks/Advisors: Bryan Garnier & Co.; Kempen & Co.; Wells Fargo Securities LLC; William Blair & Co.

#### FATE THERAPEUTICS INC.

**Fate Therapeutics Inc.** netted \$141.5m through a public sale of 8.6 million common shares at \$17.50. The company will use the proceeds to support development and manufacturing of its next-generation cellular immunotherapies for cancer and immune disorders. (Sep.)

Investment Banks/Advisors: Cantor Fitzgerald & Co.; Citigroup Inc.; Jefferies & Co. Inc.; SVB Leerink; Wedbush PacGrow Life Sciences; Wells Fargo Securities LLC

#### FREQUENCY THERAPEUTICS INC.

**Frequency Therapeutics Inc.** (regenerative medicines) filed for its initial public offering. (Sep.)

Investment Banks/Advisors: Cowen & Co. LLC; Goldman Sachs & Co.; JP Morgan & Co.; Mizuho Bank Ltd.

#### IGM BIOSCIENCES INC.

**IGM Biosciences Inc.** (engineered IgM antibodies for cancer) netted \$187m through its initial public offering of 12.6 million common shares (including the over-allotment) at \$16. The company originally intended to sell 7.8 million shares at a range of \$15-17. (Sep.)

Investment Banks/Advisors: Guggenheim Partners LLC; Jefferies & Co. Inc.; Piper Jaffray & Co.; Stifel Nicolaus & Co. Inc.

#### OPTINOSE INC.

**OptiNose Inc.** (focused on the ENT and allergy spaces) entered into a debt financing of up to \$150m with Pharmakon Advisors. Optinose initially sold \$80m five-year senior secured notes. It will issue another \$30m notes by February

15, 2020, another \$20m in 2020, and \$20m in 2021. The tranches will be based on achievement of revenues tied to its *Xhance* (fluticasone propionate nasal spray) for chronic sinusitis. OptiNose will use the proceeds to repay and retire existing senior secured notes with Athrium Opportunities. (Sep.)

#### PLUS THERAPEUTICS INC.

**Plus Therapeutics Inc.** (cancer drug development; formerly Cytori) netted \$13.9m through a public offering of 289k common shares at \$5 and 2.7 million pre-funded series V warrants (exercisable for 2.7 million common) at \$5. Investors also received five-year series U warrants to purchase a total of 3 million common shares exercisable at \$5. (Sep.)

Investment Banks/Advisors: HC Wainwright & Co.

#### PROVENTION BIO INC.

**Provention Bio Inc.** netted \$37.6m through a public offering of 5 million common shares at \$8. The company is developing therapeutics for immune-mediated diseases and will use the proceeds to continue work on its pipeline projects for conditions including diabetes, inflammatory bowel disease, celiac disease, lupus, and acute coxsackie B virus. Concurrent with the public stock sale, Provention also announced that it received a \$20m private equity investment from strategic partner **Amgen**. (Sep.)

Investment Banks/Advisors: Cantor Fitzgerald & Co.; HC Wainwright & Co.; SVB Leerink

#### PROVENTION BIO INC.

**Provention Bio Inc.** (treatments for immune mediated diseases) grossed \$20m through a private equity investment made by **Amgen**, which purchased 2.5 million common shares at \$8 apiece. The financing (announced concurrent with a 5 million share public offering completed by Provention) was carried out under the terms of a celiac disease-focused license agreement penned last November between Amgen and Provention. (Sep.)

#### PTC THERAPEUTICS INC.

Rare disease-focused **PTC Therapeutics Inc.** sold \$250m aggregate principal amount of its 1.5% convertible senior notes due 2026 to qualified institutional buyers. The notes convert at an initial rate of 19.0404 shares of common stock per \$1k principal amount, or \$52.52 per share (its stock is currently averaging \$43.07). (Sep.)

#### PTC THERAPEUTICS INC.

Rare disease drug developer **PTC Therapeutics Inc.** netted \$97.3m in its follow-on public offering of 2.48 million common shares at \$40.40 each. The

company will use the proceeds for R&D activities. (Sep.)

Investment Banks/Advisors: Credit Suisse Group

#### SATSUMA PHARMACEUTICALS INC.

**Satsuma Pharmaceuticals Inc.** (developing nasally delivered migraine therapeutics) netted \$76.7m in its initial public offering of 5.5 million shares at \$15, the mid-point of its anticipated range. The company originally planned to sell 5 million shares. (Sep.)

Investment Banks/Advisors: Credit Suisse Group; Evercore Partners; SVB Leerink

#### SPRINGWORKS THERAPEUTICS INC.

**SpringWorks Therapeutics Inc.** (developing treatments for cancer and rare diseases) netted \$173.3m through its initial public offering of 10.35 million common shares (including the over-allotment) at \$18. The company originally intended to sell 7.35 million shares at a range of \$16-18. (Sep.)

Investment Banks/Advisors: Cowen & Co. LLC; Goldman Sachs & Co.; JP Morgan Chase & Co.; Wedbush PacGrow Life Sciences

#### TRANSLATE BIO INC.

**Translate Bio Inc.** (develops messenger RNA therapeutics to treat diseases caused by protein or gene dysfunction) netted \$84.6m in a follow-on public offering at 9 million common shares at \$10 each. The company will use some of the proceeds for ongoing development of Phase I/II MRT5005 for cystic fibrosis and for additional pipeline candidates. (Sep.)

Investment Banks/Advisors: Citigroup Inc.; Jefferies & Co. Inc.; SVB Leerink

#### TURNING POINT THERAPEUTICS INC.

**Turning Point Therapeutics Inc.** (targeted solid tumor treatments) netted \$190m through a public offering of 4.5 million common shares at \$45. The company made its public debut earlier this year selling 10.6 million shares at \$18. Proceeds from the current offering will support upcoming Phase II studies with lead candidate repotrectinib for advanced solid tumors, and will also help further development of additional pipeline projects. (Sep.)

Investment Banks/Advisors: Goldman Sachs & Co.; Guggenheim Partners LLC; SVB Leerink; Wedbush PacGrow Life Sciences

#### UNIQUIRE NV

**UniQure NV** (gene therapies for hemophilia, Huntington's disease, and other severe genetic diseases) netted \$211.5m through a public offering of 4.9 million ordinary shares at \$46. Proceeds will fund completion of development activities for AMTo61 (etranacogene dezaparovec; Phase III for hemophilia

B); continued work on AMT130 (entering Phase I for Huntington's disease); and additional pipeline projects/working capital. (Sep.)

Investment Banks/Advisors: Cantor Fitzgerald & Co.; Goldman Sachs & Co.; HC Wainwright & Co.; SVB Leerink; Stifel Nicolaus & Co. Inc.; SunTrust Banks Inc.

#### VBI VACCINES INC.

**VBI Vaccines Inc.** netted \$37.8m through a public offering of 80.5 million common shares (including the overallotment) at \$0.50. The company is developing vaccines for infectious diseases and immunoncology, and will use the proceeds to continue work on pipeline projects *Sci-B-VAC* (Phase III in the US, EU, and Canada for hepatitis B; already approved in 10 other countries), VBI1901 (Phase I/IIa for glioblastoma multiforme), VBI2601 (pre-clinical, hepatitis B), and VBI1501 (Phase I completed for cytomegalovirus). (Sep.)

Investment Banks/Advisors: National Securities Corp.; Oppenheimer & Co. Inc.; Raymond James & Associates Inc.

#### ZEALAND PHARMA AS

Peptide therapeutics developer **Zealand Pharma AS** raised DKK559.6m (\$82.2m) through the private placement of 3.98 million new shares at DKK140.79 each (a 4% premium) to Van Herk Investments. The company will use the proceeds for ongoing R&D activities and to launch its first fully owned asset. Morgan Stanley was the placement agent. (Sep.)

Investment Banks/Advisors: Morgan Stanley & Co.

### RESEARCH, ANALYTICAL EQUIPMENT & SUPPLIES

#### FINANCINGS

#### 10X GENOMICS INC.

Life science technology company **10x Genomics Inc.** netted \$417.1m in its initial public offering of 11.5 million Class A common shares (including the overallotment) at \$39. The company originally intended to sell 9 million shares between \$31-35, later upped to a \$36-38 range. (Sep.)

Investment Banks/Advisors: Bank of America Merrill Lynch; Cowen & Co. LLC; Goldman Sachs & Co.; JP Morgan & Co.

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