2018 a hard act to follow; catching an innovation wave in 2019 and beyond

By Karen Pihl-Carey, Analyst

PHILADELPHIA – Record new molecular entity approvals, record venture capital financings, record Chinese investments and record research-stage acquisitions all leave biopharma executives attending the Biotechnology Innovation Organization’s international convention with one pressing question.

What’s next for innovation?

While 2018 was a stellar year for the industry, it is not a predictor for a perfect future, and current numbers indicate 2019 is not on target to hit the record levels of last year. Yet panelists at a Super Session on Monday afternoon suggested a number of ways to stay ahead of the innovation wave, such as focusing on digital technologies and tools, as well as cell and gene therapies, and presenting investors not only with a clear understanding of mechanism, but the ability to see beyond present opportunities and into expanded market potential.

“Think what you’re going to see is a revolution in the data that allows you to actually understand what’s going on at a biological level,” said Benjamin Thorner, senior vice president and head of business development and licensing for Merck & Co. Inc., of Kenilworth, N.J. “If we look backwards, we have a number of medicines that have had an impact on patient’s lives, but I think we’re really at the dawn of being able to understand how all of these mechanisms actually work inside the human body. And I think the tools that we have today, whether they be computing tools or whether they be biological tools, just unlocks an extraordinary opportunity for developing new therapeutics.”

David Thomas, BIO’s vice president of industry research, reported on the organization’s 5th annual report on emerging company trends, which included data sourced from Clarivate Analytics, the parent company of BioWorld. The industry hit several records in 2018, including the 59 new molecular entities approved by the FDA. Thomas said he expects this year to “not be as high as last year,” maybe around 40, based on the 11 already approved so far. The approvals consist mostly of small molecules as opposed to large molecules, although the second gene therapy reached U.S. approval just a few weeks ago.

As for clinical pipelines, there are currently almost 7,000 ongoing drug programs with 73% of them coming from small companies, he said. Since BIO 2018 in Boston, the industry has netted 305 new programs, most focused on oncology, although the metabolic space experienced the highest year-over-year gain of 20%.

Sam Ulin, a managing director with Newton, Mass.-based Clearview Healthcare Partners, said the huge interest in oncology is mainly driven by CAR T therapies. “From our vantage point, a lot of opportunity is outside of oncology,” Ulin said. “Companies working outside of oncology are still driving tremendous value.”

Public funding, including a total 47 biotech IPOs in 2018 tracked by BIO, showed an end-of-year market capitalization average of $710 million, according to Thomas. That compared with a $428 million average 10 years ago.

It was also a record year last year of $43 billion for research-stage acquisitions, Thomas said, with a handful making up the bulk of the money, including Celgene Corp.’s acquisition of Juno Therapeutics Inc. for $9 billion, Novartis AG’s takeover of AveXis Inc. for $8.7 billion, Sanofi SA’s buyout of Ablynx NV for $5.1 billion and Novartis AG’s purchase of Endocyte Inc. for $2.1 billion. Thomas noted that the volume of deals has decreased, meaning the average acquisition cost for a phase III company has risen significantly.

Venture capital poured $17.5 billion in therapeutic-only companies last year, with the top 15 venture raises in the U.S. each bringing more than $100 million for $3.5 billion total. For ex-U.S., almost all of the top 15 raised above $100 million, and 10 of them came “out of China,” Thomas said. “That’s where we see some of the largest venture rounds for ex-U.S.”

China was only 1% of the ex-U.S. venture capital in 2009, compared with 47% in 2018, and a large amount of the money is going into preclinical-stage companies.

Series A financings globally in 2018 raised the highest amount ever, Thomas said, and there have been significantly more licensings – 29 in total – rising above $1 billion than ever before.

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Broken down, those deals include 13 for monoclonal antibodies, seven for small molecules, nine for monoclonal antibodies/bispecifics, five for antisense therapeutics, five for RNAi, five for cell therapy, and the rest for peptides, protein conjugation, mRNA, gene editing, gene therapy and others.

Ulin said that the willingness a few years ago to bet on differentiated technologies, such as cell therapies and genome editing technologies, did not really exist, “whereas today, it is arguably the single biggest driver of investment. People aren’t shy, at least in today’s capital market, about supporting those investments.”

A half dozen years ago, it was “difficult to engage in partnering an immuno-oncology asset,” Thorner said, but once the promise was evident, it led the industry to invest dramatically in “something that had an outsized potential.”

Seeing beyond the current opportunity is paramount, such as the potential for immuno-oncology assets to be used in different therapeutic areas as more knowledge is gained of the immune system, both innate and adaptive.

“Some of the approaches in the rare disease area are ways to pry open our larger unmet medical needs,” said Barbara Dalton, a senior managing partner of Pfizer Ventures, a unit of New York-based Pfizer Inc.

While venture capital money flowing into the biopharma space in 2018 hit record levels, some new sources of capital are coming from tourists rather than residents, Dalton said.

“Usually areas of the globe start investing heavily and then they disappear,” she said. “We’re in a great place right now, but please don’t spend all of your seed in corn.”

At Pfizer, she takes a portfolio approach, investing in high-risk and low-risk opportunities, some tools and some revenue-generating companies, realizing not all of them will succeed. It is nearly impossible at the time of investment to predict what will come, but historically it has taken 25 years for companies to find success with certain antibodies and with RNAi.

“You have to have enough diversity in order to catch a wave or be in front,” Dalton said.
Platform plays offer opportunity, potential pitfalls

By Michael Fitzhugh, News Editor

PHILADELPHIA – Big scientific goals, grand partnering ambitions, and a strong tolerance for risk are common hallmarks among the CEOs and founders of emerging platform companies. But what sets platform players apart from other biopharma ventures hasn’t always been clear. Setting out to shed some light on the subject during the BIO convention, BioWorld asked the leaders of six dynamic companies about how they think about their businesses and what sets them apart.

“I hear the word ‘platform’ being used, overused, and misused all the time,” said Yochi Slonim, CEO and co-founder of Anima Biotech Inc. “To me, the most fundamental test for the word ‘platform’ is that you have something that is really applicable across many therapeutic areas.

“The difference that makes a difference” for companies that define themselves as platform players, he said, is not about an ability to generate assets, but rather about the power to provide potential partners a new strategy to develop their own assets.

Bernardsville, N.J.-based Anima did exactly that in a $1.1 billion deal with Eli Lilly and Co., announced in July 2018. The agreement, which included $30 million up front and $14 million in research funding, is for the discovery and development of translation inhibitors for several target proteins by using Anima’s translation control therapeutics platform.

The notion of a platform further connotes “a scalable unique technological asset,” said Dietrich Stephan, CEO and founder of the Pittsburgh-based rare genetic disease-focused company Neubase Therapeutics Inc. That uniqueness can take the form of, at one extreme, purely theoretical scientific insights, and at the other, substantial clinical data showing target engagement in multiple indications. Neubase is somewhere in the middle, he said, with academic data in hand showing that its gene silencing platform, Patrol, can have an impact in a variety of indications, and efforts are underway to generate more proof of its potential.

A little further down the road is Nurix Inc., a San Francisco-based company developing drugs to modulate ubiquitin E3 ligases, key enzymes responsible for protein breakdown in human cells using two complementary platforms. In 2015, it landed a broad collaboration with Celgene Corp. (now part of Bristol-Myers Squibb Co.) around targeting protein homeostasis for therapies in oncology, inflammation and immunology.

Despite securing that success, very few platforms can be monetized effectively, said Arthur Sands, CEO of Nurix. “They have to be extremely exciting and capture the popular press, like CRISPR and genomics did,” he said. Combining a platform’s story with therapeutic one can help, too, Sands said. Not to be forgotten, “you also need a lot of cash to survive,” he said.

Broadening out

Of course, not every platform company began that way. Immusant Inc., of Cambridge, Mass., got its start developing an antigen-specific immunotherapy for Celiac disease, Nexvax2, still its lead candidate. But when CEO and president Leslie Williams saw the potential for the startup’s technology to be applied more broadly to other autoimmune indications, such as type 1 diabetes, she and her team began to explore “how to unravel autoimmunity,” she said. That move has taken its technology to a higher new level with a key attendant benefit, she said: “If we had not done that, we would never get the value that we are now able to generate with our core program.”

Yet even with diversification, platform players face some of the same high-stakes moments other companies do. Managing them can take careful planning, something Williams is embracing now ahead of an end-of-year readout for a phase II trial of Immusant’s Nexvax2. “The science is going to carry the day,” she said, but her team isn’t taking any chances, building into its trial not just a primary endpoint, but also “a whole plethora of data that could really support the platform and how it works,” she said.

Plenty of external risk remains, too, of course. When other companies start working on the same approach, it can be a double-edged sword, said Filippo Petti, CEO of Mont-Saint- Guibert, Belgium-based Celyad SA. “You are swimming upstream when you go out and talk about a novel platform. So, to have others validate that, maybe with their own details behind it, can help strategic investors understand the platform, he said.

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Other components of platform risk management, such as decisions around the locus of development work and manufacturing, can be less clear. Anima’s Slonim pointed out the importance of working on targets from partners within one’s own company, noting that “otherwise, it's a very very dangerous game, where you may be accelerating the devaluation of your platform.”

Yaky Yanay, president and co-CEO of Haifa, Israel-based Pluristem Therapeutics Inc. took a similar view, noting that contract manufacturers would love to get their hands on the company’s allogeneic cell therapy technology. Instead, Pluristem is keeping a close hold on its manufacturing because his team understands that once they give it up, it would be gone.

Ultimately, he said, “you have to be the best in the world in actually being able to apply your platform.” Accomplishing that is likely a challenging but lasting key to lasting success.
BIO 2019: Welcome to ‘Cellicon Valley’

Cell and gene therapies will be showcased at Philadelphia meeting

By Peter Winter, BioWorld Insight Editor

Philadelphia has not been traditionally known as a leading hub for biotechnology innovation and commercialization, in contrast to those hubs located Boston and San Francisco. However, the city is hoping that the more than 16,000 delegates attending the BIO 2019 International Convention this week will learn just how much research and development is taking place and that global recognition for its foundational research in gene and cell therapy has earned it the name of “Cellicon Valley.”

Certainly, the number of biotechnology companies located in the greater Philadelphia region has grown rapidly during the past few years and, according to the CEO Council for Growth, a council of the Chamber of Commerce for Greater Philadelphia, the region is now home to more than 30 cell and gene therapy companies.

Jim Greenwood, president and CEO of the Biotechnology Innovation Organization (BIO), told BioWorld Insight that one of the interesting things about this year’s meeting is that attendees will gain an appreciation of the tremendous resurgence of gene therapy that has taken place in the area. That has evolved after a very difficult start almost 20 years ago when a young patient died as a result of the experimental therapy he received at the University of Pennsylvania.

The 1999 death of 18-year-old Jesse Gelsinger came four days after researchers injected genes to cure ornithine transcarbamylase deficiency, a genetic metabolic disease. (See BioWorld Today, Dec. 10, 1999.)

The technology remained in the doldrums after that serious setback but slowly it has got off the floor and flourished thanks to breakthrough discoveries in cell and gene therapies, much of which originated in the region. That seminal work has culminated in several new medicines reaching the market, including Philadelphia-based Spark Therapeutics Inc.’s FDA approval for Luxturna (voretigene neparvovec-rzyl), which became the first gene therapy approved in the U.S. that targets an inherited disease caused by mutations in a specific gene. The treatment was approved to treat children and adults with confirmed biallelic RPE65 mutation-associated retinal dystrophy, an ultra-rare progressive disease that leads to vision loss and may cause blindness. (See BioWorld, Dec. 20, 2017.)

Despite the uncertainties of the capital markets during the past 12 months, public and private biopharma companies have had no difficulty raising capital – almost $58 billion, in fact, generated from 1,049 transactions.

Unique period

Along with those developments, the pace of discovery continues in all areas of biotechnology. It is a unique period right now for the industry, Greenwood reflected, where the science has never been better but, unfortunately, at the same time, the prevailing political climate has never been more challenging as the debate on the current state of drug pricing rages on.

Those headwinds are likely to keep battering the industry and intensify as the upcoming 2020 U.S. presidential election campaign gets into full swing.

BIO 2018 – BIO 2019

Although Wall Street has acknowledged the political risk that swirls around the industry, it believes, for the time being, that something radical is not about to happen. However, “it won’t take much, in terms of bad policy related to health care, to spook investors,” Greenwood said.

Since BIO 2018 closed it doors in Boston last June, public biopharmaceutical companies have experienced some good times as well as low periods, particularly in the fourth quarter of 2018 when investors shied away from biopharma company equities big time. As a result, the BioWorld Biopharmaceutical Index dropped almost 7% in December. The month will also go down in history as one of the worst periods for equities, with the Dow Jones Industrial Average stumbling by almost 9% and the Nasdaq Composite performing just as poorly, dropping 9.5%. (See BioWorld Insight, Jan. 7, 2019.)

Fortunately, the early months of this year saw investors return to the sector, anticipating that there will be an increase in the number of M&A transactions going forward. That belief

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was certainly reinforced with Roche Holding AG’s acquiring Spark Therapeutics, paying $114.50 per share in an all-cash deal valued at about $4.8 billion. Spark will operate as an independent firm inside Roche after the deal is completed. (See BioWorld, Feb 26, 2019.)

Over the past 12 months, the positive and negative swings in capital market performance have balanced out, with the BioWorld Biopharmaceutical Index closing the period down just 1% compared to the Dow Jones Industrial Average and the Nasdaq Composite index dipping 3% and 1.7%, respectively. (See BioWorld Biopharmaceutical Index BIO 2018 – BIO 2019, below.)

Turbulent markets hit drug developers

While there has been a resurgence of investor interest this year in small and midcap drug developers, the turbulent markets have hit those biopharmaceutical companies. “Generalist investors fear the constant scrutiny of the industry’s pricing practices and negative headlines as the Presidential primaries approach and so are looking for ideas in less-controversial sectors. They have not participated in any meaningful way in biotech year-to-date in 2019, and appear unlikely to return any time soon,” the Cowen and Co. biotechnology analyst team wrote in its April biotech thermometer report.

Overall, in the 12-month period since BIO 2018, the BioWorld Drug Developers Index recorded a 21% drop in value. (See BioWorld Drug Developers Index BIO 2018 – BIO 2019, below.)

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### Biopharma capital raised: BIO to BIO

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*Source: BioWorld*

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**Cash flows**

Despite the uncertainties of the capital markets during the past 12 months, public and private biopharma companies have had no difficulty raising capital – almost $58 billion, in fact, generated from 1,049 transactions, according to data from *BioWorld*. (See Biopharma capital raised: BIO to BIO, above.) Of that amount, approximately 71% was raised by companies completing public offerings, including follow-on financings and IPOs. It is notable that the turbulence experienced in the general markets did not serve to dampen the enthusiasm for IPOs, with a steady flow of biopharma company IPOs completed throughout the year. A total of 82 companies closed their offerings, generating almost $11 billion in the process, compared to the almost $6.2 billion generated in the BIO 17-BIO 18 period.

Private companies have also captured a record amount of capital. The 395 companies that have raised cash since BIO 2018 have brought in a massive $16.6 billion. This year alone global private companies attracted more than $4.4 billion in the first quarter, 10% more than the $4 billion generated in the first quarter last year. In fact, it is also the highest amount raised in this period in the past decade. That total could signal that the sector is well on its way to surpass the record $17 billion private financings set in 2018.

**By the numbers**

Since the BIO International Convention was last held in Philadelphia in 2015, the industry has been on a record-breaking pace for generating capital. That year, the high bar was set with a total of $68.4 billion raised. Last year, according to *BioWorld* data, $67.1 billion was raised by global companies from public and private sources, which places it firmly in second place in terms of totals raised in the history of the industry. (See 21 years of biopharma financings, page 4.)

The $20 billion raised this year to date means that in the past five years the global biopharma industry has generated an incredible $250 billion from its fundraising activities.

Business development has been equally robust in that period, with the value of partnering transactions averaging about $90 billion annually. (See 21 years of biopharma deals, page 4.)

The expectation by investors that mergers and acquisitions will pick up speed appear to have been confirmed, with the 2019 total of M&A transactions already outpacing the 2018 total. (See 21 years of M&As, page 4.)

**Let's make a deal**

Greenwood noted that the annual convention’s One-on-One Partnering has evolved into an important component of the BIO meeting. At last year’s event, more than 46,000 meetings were recorded, which set the Guinness World Record for the “Largest Business Partnering Event.”

The meeting in Philadelphia is already on pace to surpass that record, he said.

Those invaluable connections form the foundation of innovative industry collaborations and scientific breakthroughs and BIO has selected the theme of “It starts with one” for this year’s event. Greenwood explained that it only needs one molecule, one scientist, one conversation that can lead to the creation of one company that could create a lifesaving new medicine.
Boosting rare disease science with collaborative research; putting patient need first

By Karen Pihl-Carey, Analyst

PHILADELPHIA – Developing new therapeutics for small patient populations is a challenging endeavor for researchers, but a collective 30 million patients in the U.S. with an estimated 7,000 rare diseases continue to wait for treatments.

The National Organization for Rare Disorders (NORD) has developed a rare disease registry platform to help alleviate some of the roadblocks researchers face, such as poorly understood diseases that have phenotypic diversity and small numbers of patients that are geographically dispersed. Not only has NORD worked directly with rare disease foundations, but it has built a feature into its platform in order to help industry with clinical research. Pending a grant, the 36 registries could potentially validate patient-reported outcomes to fulfill FDA expectations.

“I think there is this wonderful culture shift where we really are focusing in on asking people what they need and are better listening and understanding what would work best for them in terms of solutions before we actually start to build the solutions,” said Vanessa Boulanger, director of research at NORD, during a session Wednesday at the Biotechnology Innovation Organization’s international convention.

Boulanger was joined by La Jolla, Calif.-based Trio Health’s CEO Brent Clough during the discussion to present The Power of Patients: Informing Our Understanding of Rare Diseases, a 140-page book that details patient stories, the overview of conditions and aggregated data from NORD’s registries for six rare diseases. The registries were developed in partnership with patient organizations, and the book's foreword is written by Janet Woodcock, director of the FDA's Center for Drug Evaluation and Research. Some of the data were presented at the American Society of Clinical Oncology meeting and other industry conferences.

Anne Pariser, director of the Office of Rare Diseases Research at the National Institutes of Health's National Center for Advancing Translational Sciences, outlined the problems with rare disease research. While rare diseases each affect fewer than 200,000 people in the U.S., “collectively, rare diseases are not actually rare,” Pariser said, and there are about 200 new diseases recognized each year. Of the 7,000 total rare diseases, about 95% have no approved treatments.

Due to a lack of disease experts, many of the conditions are misdiagnosed and patients, facing life-threatening symptoms, travel far and wide to find answers. Industry scientists that are tackling rare diseases often find no clinical trial precedents and struggle to find enough patients to support their research. Still, Pariser said that this is a “time of great opportunity” with CAR-T therapies, a second gene therapy approval for spinal muscular atrophy last week, and a record-breaking number of orphan drug approvals, as well as the potential of 3D printing, artificial intelligence, gene therapy, gene editing and RNAi therapies.

The book created by NORD and Trio Health looks deeply into patient challenges with diagnosis and treatment.

One patient featured, Michelle, was eventually diagnosed with the rare neurological disorder opsoclonus myoclonus syndrome (OMS). In 2010, at age 24, she began to experience vertigo and muscle spasms, had monthly emergency room visits, several MRIs, and saw multiple neurologists who misdiagnosed her condition. Her journey took her from her home in Oregon to New York where she finally received the OMS diagnosis four years after her symptoms first appeared.

Another patient eventually diagnosed with the rare autoimmune skin disease pemphigus/pemphigoid (P/P) had met with more than five doctors who incorrectly diagnosed him. He found the International Pemphigus & Pemphigoid Foundation on the internet and read about the symptoms. A patient advocate from IPPF recommended he go to see a dermatologist in Boston, which was a thousand miles from his home. At the first visit, he was correctly diagnosed, and then treated monthly.

“This is not a criticism of any particular physician,” Clough said. “These are difficult. These are hard-to-treat patients and hard to discern,” considering the commonality of the symptoms.

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Another major stumbling block for patients with rare diseases is finding the right treatment, including those off-label. The book highlights the types of therapies available and the percentage of patients receiving certain medications. Quality of life, as well as physical and mental health, are all important considerations for designing treatments of rare diseases. Two young sisters featured in the book, Lily and Elise, were exhibiting symptoms that doctors initially diagnosed as epilepsy, but later turned out to be Synap1-related non-syndromic intellectual disability. The book serves as a roadmap for understanding quality of life and other issues and how they are currently addressed for these small patient communities.

“We had patient organizations, we had NORD, we had the FDA and we had Trio Health, and we all came together for the good of the patient to produce what we’ve done,” Clough said.

Companies working in the rare disease space also are forming bridges with patients to help develop meaningful outcome measures.

Strongbridge Biopharma plc, of Dublin and Trevose, Pa., is focused on a portfolio of rare neuromuscular and endocrine condition treatments, and has one FDA approved product, Keveyis (dichlorphenamide) to treat primary periodic paralysis, which it acquired from another company. The 4,000 to 5,000 patients with the disease spend up to 20 years trying to get a diagnosis. Strongbridge developed a genetic testing program to try to reduce that time frame, and has worked to build awareness of the disease, making clear that patients can be genetically negative but clinically positive. Steps taken to build partnerships with the patient community “starts off with the art of listening and not going in with your own agenda and being open to listen to their concerns,” said Vanita Sharma, executive director of Patient Advocacy and Public Policy at Strongbridge. “It also goes beyond the patient; it also goes to caregivers” in understanding their needs and challenges.

Last fall, NORD added a new feature on its platform for external researchers who want to run a study in one of the communities in which there is a registry. They can partner with NORD and the advocacy organization.

“This was NORD’s solution to overcome some of the duplication and redundant registry efforts that we were seeing in the space,” Boulanger said, “and a way to keep the patient communities together to preserve the power of the data.” Boulanger recently wrapped up a few grants proposals with partners to help companies validate patient-reported outcomes, which is required by the 21st Century Cures Act.

“It’s a really great way to use the tool that we’ve developed that we haven’t quite done yet, but we’re right on that forefront,” she said, “because we recognize the need for validated tools that are specific to the rare disease community.”
Australia takes BIO ‘by storm’ with largest ever delegation

By Tamra Sami, Staff Writer

PHILADELPHIA – This year’s Australian delegation saw the most impressive participation to date at the Biotechnology Innovation Organization’s (BIO) 2019 conference, with more than 150 Australian companies participating as well as numerous government representatives.

The country has a lot going for it lately as the biotechnology and med-tech sectors have matured in the last decade, and there is more capital available than ever before. That increased activity has gotten state government attention, and those budgets are beginning to reflect new investments into the sector to continue to grow the ecosystem.

Western Australia (WA) sent its first BIO delegation, which brought the state’s Deputy Premier and Health Minister Roger Cook and Chief Scientist Peter Klinken to Philadelphia. Queensland Premier and Minister for Trade Annastacia Palaszuk also made the trek from the southern hemisphere as did Queensland Minister for Innovation and Tourism Kate Jones.

Deputy Premier Cook told BioWorld that it was his first trip to the U.S., and he was pleased that the Labor government in WA is committing more funds into medical research, which will allow Aussie biotechs to engage more with multinational pharma and med-tech companies.

Western Australian has committed AU$1.3 billion (US$908 million) to the Future Health Research and Innovation Fund to supercharge future investment in health and medical research and innovation, doubling the current annual expenditure.

“Australians have really taken BIO 2019 by storm, said MTPConnect CEO Dan Grant.

“It’s the largest delegation I can remember, and the interest in Australia’s biotech and pharma offering has been intense. In particular, the heightened awareness here of Australia as a preferred destination for clinical trials has been really pleasing to see. Our growing reputation globally for speed, quality and regulatory simplicity is truly something we can build on to grow a powerful export industry that also delivers for patients,” he added.

MTPConnect released at the conference a new report, titled “How Global MedTech and Pharma Corporates Engage with Australia,” which underscores that the market for innovation is global and reaffirms the high regard that multinational pharmaceutical and medical technology firms generally have for Australia’s excellence in science and research, Grant said.

“While there are some pragmatic barriers to further increasing our already strong engagement with these firms, such as Australia’s geographical isolation, the report pinpoints tangible
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solutions to mitigate any hurdles,” he added. “Seeking early stage collaboration to get our innovators in front of key decision-makers is important, as are focusing on areas of overlap between the multinationals and areas of excellence in Australian innovation and being at the right networking events.”

Frustration with government policy

Meanwhile, AusBiotech’s annual survey of biotechnology industry CEOs shows that while Australia’s strength in life sciences remains in a growth trajectory, there is a worrying fall in business sentiment across the sector with uncertainty over the fate of the R&D Tax Incentive (RDTI).

The Biotechnology Industry Position Survey 2019 showed business sentiment in the biotech sector is pessimistic due to a steady erosion of government support.

Only 14% described the Australian operating environment as conducive to growing a biotech business, down from 37% the year before. This is the worst result this decade and points to a challenging period ahead. Almost 26% of respondents said the environment “works against growing a biotech business,” an increase from 16% in 2018.

While industry metrics are still sound and growth still expected, the survey revealed a “wait and see” approach from companies in regard to employment, with a sharp increase in the plan to hold staff levels steady (24% to 47%) and a decrease in the intention to hire (73% to 51%). The past year saw a significant drop in those that experienced an excellent year (from 29% to 19%).

The industry’s frustration on the continued threat to the RDTI was “loud and clear,” the report said. “Both the lack of vision for industry growth levers and plans to cut support to the sector has shown a disillusionment across the industry, the likes of which we have never seen before.”

The R&D Tax Incentive is the most critical centerpiece program in the translation of Australia’s research, and the program has been successful in helping attract more investment in R&D and fostering a strong Australian life sciences clinical trials sector. “Industry is frustrated at the government’s lack of commitment to a business environment that better supports our cutting-edge research and development,” said AusBiotech CEO Lorraine Chiroiu.

“However, Australia’s strength in life sciences still shines globally and contributes nationally, economically and socially.”

Michael Cunningham, national head of life sciences, Grant Thornton Australia, which co-authored the report, said commercialization represents the greatest need to keep profits and future development opportunities in Australia. “Emerging markets in the Asia-Pacific region are making biotechnology a priority, and Australia must continue to provide an attractive landscape for life sciences firms to conduct R&D, manufacturing and domestic distribution activities to fuel growth in 2019 and beyond.”

Alongside business sentiment and policy concerns, the survey found:

• There is a prevailing view that support for tech transfer and commercialization diminishes as research leaves public institutions on the commercialization pathway.
• Poor metrics, combined with a lack of understanding of the skills and investment needed for the industry’s future, has conspired to drive poor policy decisions.
• Regenerative medicine is lighting up the horizon, with Australia preparing for a regenerative medicine revolution. This relatively new science field is expected to disrupt the health treatments available to patients worldwide.
• Medicinal cannabis has burst onto the scene. The global sentiment toward medicinal cannabis is changing, and Australian biotechnology companies are claiming the space to demonstrate the medicinal value for patients.

“It’s the largest delegation I can remember, and the interest in Australia’s biotech and pharma offering has been intense.”

Dan Grant
CEO, MTPConnect