

The New Era of Life Sciences

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The new era of life sciences has already begun. The global pharmaceutical market is expected to reach over \$1.5 trillion by the end of 2023, as cutting-edge technologies are transforming the way we understand and treat diseases, and new medicines offer hope for millions where previously there was none. From the decoding of the human genome and the advent of precision medicine, to the search for cures for Alzheimer’s and cancer, the integration of technology in healthcare within the life sciences field has spurred remarkable progress in recent years. However, the industry also faces several regulatory and reputational challenges, notably due to the high prices of life-saving treatments and policy changes that some claim will hamper innovation. At the same

time, the Covid-19 pandemic has served as a turning point for the way the public perceives the sector. Pharmaceutical companies have stepped up to the plate to develop life-saving vaccines and treatments at a rate that has been nothing short of a marvel, showing that when the industry puts its focus on public health the results can be impressive. In this special report, we present the culmination of diligent research: the result of comprehensive interviews with a distinguished cohort of decision-makers who shape the future of the industry. Our interviewees consist of North America’s leading CEOs, association leaders, and government officials. Amidst the many transformative developments, there is no doubt that the impact of these innovations and policies will be significant, shaping the way we all live our lives. ▶

In This Report...



ALBERT BOURLA | CEO, PFIZER
We are living through a scientific renaissance – fueled by advancements in biology and technology. The years ahead will have a dramatic impact on human health.



LEONARD SCHLEIFER | CEO, REGENERON
Prices are higher in the U.S. because payers have been willing to pay the price of innovation; this is the reason we have certain medicines at all in the first place.



PIERRE FITZGIBBON | ECONOMY & INNOVATION MINISTER OF QUEBEC
If we correlate the national health system database with the immense power of AI, we have the potential to develop some strong, and even revolutionary solutions.



STEPHEN UBL | PRESIDENT & CEO, PhRMA
Changing or altering people’s genes to cure them used to be the stuff of science fiction, but we are making it a reality.

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AUDREY GREENBERG

CO-FOUNDER
CBM

(Center for Breakthrough Medicines)

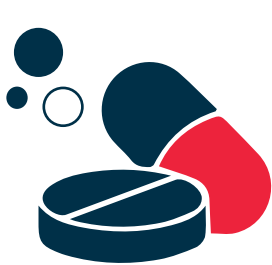


2022 was a record year for cell and gene therapy approvals, and 2023 is set to surpass it by five times. CBM partners with clients to streamline production and optimize capital deployment to allow innovators to focus on research while we deliver expertise and capacity.

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From DNA to Data

The life sciences industry is shifting ever more from one-size-fits-all treatments to individual, tailor made cures. This is made possible through the science of genomics, one of the most exciting and rapidly advancing fields in the life sciences. In brief, within the genome all genetic information that plays a dominant role in determining the traits and characteristics of an organism are encoded: the recipe for all life itself. In the past 30 years, unprecedented advances in DNA sequencing technologies have made it possible to analyze vast amounts of genetic data. These insights into the underlying mechanisms of disease through genomics have revealed new avenues for the development of personalized treatments.



\$1.5 trillion

estimated value of the global pharmaceutical market by the end of 2023

Today it is possible to sequence entire genomes at a much faster pace and lower costs more than ever before, thanks to the efforts of innovators such as Illumina. Its CEO, Francis deSouza, tells us that “it cost \$3 billion and thirteen years to sequence the first single human genome in 1990. When we introduced our first sequencer in 2007, the price was around \$150,000, and between then and 2023 we brought the price down to \$200 - which means we have lowered our prices by 99.9%.” The benefits of such advancements are manifold, impacting areas ranging from gut health to rare diseases - and, according to deSouza, even information science: “There is even research being done today about how we could use DNA as a storage medium for artificial data, given that it is much more efficient than current hard-disks.”

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Pushing Boundaries



DEBBIE HART | PRESIDENT & CEO, BioNJ
What is quite astonishing is the fact that people now live on average 10 years longer than they did in 1989. Meanwhile the global pharmaceutical industry invested an unprecedented \$200 billion in R&D in 2020.



PAMELA FRALICK | PRESIDENT, INNOVATIVE MEDICINES CANADA (IMC)
Canada is a nation of settlers, along with our Indigenous populations. As a result, we're able to conduct clinical trials on a broad, diverse population, especially in larger cities like Montreal, Toronto, and Vancouver, which is extremely attractive for the innovative pharmaceutical industry.



KEREN HARUVI SNIR | PRESIDENT, SANDOZ U.S.
According to our studies, by 2025, 1.2 million additional patients could get access to biologics just by virtue of the fact that we will have a greater volume of biosimilars available.



JOE PANETTA
| PRESIDENT & CEO, BIOCOCALIFORNIA
We believe opportunity exists for collaboration between California-based companies and other markets. Biocom California has an office in Tokyo, and we plan to support our members who wish to expand to Asia and Europe.



RENE RUSSO
| CEO, XILIO THERAPEUTICS
We believe that cancer treatment can be revolutionized through medicine that effectively tricks tumors into activating their own treatment, while protecting the patient's healthy tissue, organs, and cells.

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DR. FRANK BEDU-ADDO
CEO
PDS Biotech



Immunotherapies such as our Versamune® platform products that can activate large quantities of tumor-attacking killer T-cells, and also our novel tumor-targeting cytokine are key advancements in cancer treatment. We're excited to advance our lead candidate into a Phase 3 global registrational trial later this year.

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Precision Medicine & Cancer Immunotherapies

These advances and cost reductions in genomics have paved the way for the development of precision medicine: treatments tailored to the unique genetic profile of patients. VP of Genomic Health at 23andMe, Noura Abul-Husn, states that “the push of people seeking genetic information on their own is a driving force behind personalized medicine,” something unimaginable only 20 years ago when the first project to sequence the human genome was finally completed. Unlike traditional approaches, as Sadik Kassim, CTO of Genomic Medicines at Danaher tells us, personalized medicine helps to “identify the mutations that could cause diseases, and intervene with maximum precision and swiftness to correct or modify the genetic cause.” This opens doors for medical professionals to design treatments that are more effective and less toxic. ▶



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**\$2.6
billion**

estimation for the average
cost of developing a single new drug

Precision medicine is already having an impact on the treatment of cancer. Olga Potapova, Founder and CEO of Cureline, claims that “targeted therapy is a revolutionary success for the industry - it prolongs life and improves its quality by paying attention not only to the illness, but also to the patient.” Life Sciences Washington’s CEO and President, Marc Cummings, highlights these advancements while also warning about costs: “Looking ahead, I think we will see dramatic changes in cancer survival rates. It is inspiring to witness that we already have the tools to develop the next generation of therapies, but we need to bring costs down as personalized cancer treatments are often very expensive.”

Alongside immunotherapy, precision medicine is one of the greatest promises in the future of the field. Through immunotherapy, researchers have been unlocking the secrets of the immune system and discovering new ways to harness its power to fight against cancer. These therapies have shown remarkable efficacy in shrinking tumors and improving survival rates in patients who have not responded to traditional chemotherapy. CytomX’s CEO, Sean McCarthy, says checkpoint inhibitors - a type of immunotherapy - “were discovered by blocking a protein called CTLA-4, which leads to the immune system being re-awakened to see the cancer and attack it. This is the amazing advance that transformed oncology research and subsequently helped many thousands of patients around the world.” It is precisely by virtue of these advancements that biotechs like PDS could develop Versamune, a promising T-cell activating platform. The company’s early clinical trials showed that “of 29 patients who, historically, had a median survival of only three to four months to live, we managed to extend this period to 21 months, with a response rate of 63% in the group of patients who received the most effective doses from the current reported standard of less than 10%,” said Frank Bedu-Addo, CEO. ▶



CUONG DO
PRESIDENT & CEO
BioVie



What we realized is plaques and tangles were not necessarily the toxic agent causing neuronal death. What we did learn is that they are both **inflammatory**.

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BioViePharma.com
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CRISPR: The End of Genetic Disease?

Another revolutionary development in the field of genetics, CRISPR allows the DNA of living organisms to be edited with unparalleled accuracy and ease. With this breakthrough in-hand, humanity can realistically aspire to cure genetic diseases, create more resilient crops, and even design new organisms with custom properties. In the words of Trevor Martin, the CEO of Mammoth Biosciences, “what makes CRISPR really exciting is that it allows us to move from treating diseases to curing them. Rather than receiving treatment for the rest of your life you can access this one-time, potentially curative therapy that goes to the root cause of the disease.”

CRISPR works by utilizing a molecule called RNA - which may sound familiar from the Covid-19 pandemic - that acts like a guiding system to direct a special enzyme to specific locations within the DNA, which allows one to remove, add or replace genetic material with almost absolute precision. “The rise of genomic and especially CRISPR technology completely shifted the paradigm in pharma; barriers were crossed and a new pool of opportunity developed,” says John Leonard, President and CEO of Intellia. Martin even claims that CRISPR’s presence implies a philosophical transformation: “We can make changes to our DNA rather than shooting in the dark and hoping that the molecule will affect the gene. If you cure one disease it makes it easier to tackle others, because you have already figured out delivery, dosage, etc., and all you are doing is switching up the guide RNA, a very reproducible and effective method. It simply changes the game of how we do drug development. There are 4,000 genetic diseases that we could target through our work.” CRISPR might also open new avenues of treatment for autoimmune diseases. These maladies are one of the biggest challenges in medicine due to their complex and multifaceted nature, and it is precisely by editing the genes that trigger the immune system to attack its own cells that CRISPR can revolutionize the way we understand and treat autoimmune conditions.



JOHN F. CROWLEY
EXECUTIVE CHAIRMAN
AMICUS THERAPEUTICS



Biotechnology is just a great big word that means hope.

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The technology and infrastructure needed to make this a reality is still in its early days, however. Audrey Greenberg from the Center for Breakthrough Medicines enthusiastically tells us that “we are only in the early days of CRISPR technology, gene editing and allogeneic cell therapy - and they are already producing incredible results,” but Greenberg also highlights that “these innovations still remain extremely expensive.” Amongst other challenges, science has limited expertise in genomic analysis - we do not know what every specific gene ‘does’ yet - and difficulties in integrating these vast amounts of data into clinical decision-making. Additionally, some have voiced reasonable concerns apropos of how genetic data will be handled in terms of privacy and security. Yet, the exponential rate at which these technologies are advancing, make them the potential basis for a revolution that could save countless lives.



GIOVANNI CAFORIO | CEO, BRISTOL MYERS SQUIBB

Precision medicine is definitely one of the highlights of today's biotech market. If in the past cardiovascular treatments were addressed to a large number of patients, today our drug Camzyos can address genetic cardiomyopathy in a deeply individualized way.



MORITZ HARTMANN
| GLOBAL HEAD INFORMATION SOLUTIONS, ROCHE

Since the early 2000's we have been focusing on precision medicine as we have seen how the rise of genomics drove the emergence of therapies that pre-identify if patients can benefit from a certain treatment.



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Alzheimer's: Approaching a Cure

As conspicuous as the rise of genomics is in the life sciences, it is certainly not the only area where innovation seems to have reached an inflection point. In recent years, the scientific community has made great strides in unraveling the mysteries of age-related neurodegenerative diseases, including Alzheimer's and Parkinson's, which jointly affect more than 60 million people worldwide. Martin Tolar, CEO, Alzheon says that "up until last year, all Alzheimer's research has been unsuccessful, with the cost of failures over the past decade reaching tens of billions of dollars." However, new breakthroughs in research are bringing hope to those affected. Advances in genetics have provided a deeper understanding of the complex risk factors that contribute to these conditions, while developments of new drugs are offering new pathways for treatment.


60 million people worldwide
suffer from Alzheimer's and Parkinson's

Eli Lilly is targeting plaque with its drug Donanemab, which according to their CEO, David Ricks, "is a super high affinity molecule that gets rid of plaque in the human brain at a very rapid rate, which we think is the negative agent causing the trouble." Ricks also remains convinced that the study is particularly promising. BioVie, on the other hand, is one of the smaller biotechs exploring untrodden roads in relation to Alzheimer's. Its CEO, Cuong Do, tells us that not long ago "we realized plaques and tangles were not necessarily the toxic agent causing neuronal death. What we did learn is that they are both inflammatory." This means that chronic inflammation may play a significant role in the development and progression of the disease. BioVie is exploring anti-inflammatory treatments as a potential way to slow or stop the progression of Alzheimer's, and its candidate awaits FDA approval. Cuong Do also claims that inflammation is "the root of many other evils", as it "leads to the hyper-methylation of our DNA and hence an acceleration of the aging process." His advice if we are to avoid it? "Exercise better, eat better, and be careful about the environment you put yourself in."

The Macro Reality of Microbiota

Several studies are now suggesting that the gut microbiome, a collection of over 100 trillion microbes that reside in the digestive tract, is more important than we ever imagined, and potentially interwoven with many of the ailments discussed in this article. The microbiome has been shown to have a profound impact on a wide range of physiological processes, from digestion to immune function and mental health. French company Biocodex was one of



**CHRISTOPHER MOLLOY | PHD, CHANCELLOR
EMERITUS, RUTGERS UNIVERSITY**

The study of the human microbiome and potential implications of human microbiome disturbances will be a crucially important area of research in the coming decades.



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MARTIN TOLAR
CEO
ALZHEON



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the first to intuit that something of great relevance was hiding in our bacteria; "founded back in mid-50's, our founders have always felt that gut health held far more than meets the eye" says CEO Nicolas Coudurier. Marie-Emmanuelle Le Guern, VP Research & Development, tells us that "recent data have provided compelling evidence that the gut microbiota of patients with Parkinson's, depressive disorders or autism spectrum disorders (ASD) show a different composition compared to healthy people." According to Le Guern, "It seems that the "western modern diet" is an example of perturbation that leads to minor microbiota alteration." Other agents pointed at lately have been the abuse of antibiotics, which data suggest kills some of the gut's beneficial bacteria as mentioned by the Biocodex Microbiota Institute. 



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Innovation in Rare Diseases

Rare diseases in the U.S. are defined as conditions which affect less than 200,000 people. Despite their name, 1 in 10 U.S. citizens suffers from one of the 8,000 rare diseases. Recently, with the support of the FDA, researchers and pharmaceutical companies have been able to bring innovative treatments to the market that have given hope and relief to patients who previously had limited options. “Rare disease innovation has been the success story of the last two or three decades,” says William H. Lewis, Chair & CEO of Insmmed.

300 million people
worldwide are affected by one of the 8000 known rare diseases

Nowadays, more and more companies are doing research in this field. Acasti is a specialty pharma whose strength lies in reformulating already marketed drugs. Its President and CEO, Jan D’Alvise, claims “they will have two drugs ready to move into Phase 3 in 2023, and a third drug which is a topical spray used for a pain indication for a disease called postherpetic neuralgia (the single largest cause of suicide in people over the age of 70 just due to the severe pain it causes) going into Phase 2.” Another small biotech, PTC Therapeutics, is already successfully commercializing a drug that targets Duchenne muscular dystrophy, “the first approved gene therapy in the world directly injected into the brain and the first approved oral treatment for spinal muscular atrophy developed from PTC’s Splicing platform,” explained CEO Stuart Peltz. The 25-year-old company attributed its success to innovation, adding that “in this day and age, you definitely do not want to make the best horse and buggy when we have flying cars.”

Partnering for Success: Tech & Contract Outsourcing

The convergence of the tech and life sciences industries has been a story of unlikely but transformative collaboration. What once was a relationship characterized by little more than the implementation of basic software solutions in healthcare facilities has now blossomed into a far-reaching partnership, whereby data management, AI, and cloud computing are utilized to address some of the most pressing challenges in healthcare. “Digital medicine has reached a tipping point: algorithms, software programs, and phone apps have proliferated, and now make medical decisions for patients themselves,” says Stephen Perry, CEO of Kymanox.

The advent of AI, as recently encapsulated by ChatGPT, made many of us realize its mysterious power. Beyond chat boxes, however, AI is to play a vital role in drug discovery. As Sean McClain, the CEO and Founder of Absci, tells us “the industry is still trying to wrap their head around AI and how it can be applied to drug discovery and development to ultimately increase success rates and get medicines to patients faster.” One example of AI’s application in the field comes from the Canadian biotech AbCellera. Its CEO and co-founder, Carl Hansen, shares that they “have spent the last decade working to build an engine that can bring new antibody medicines to the clinic with greater speed and probability of success.” Simultaneously, IT giants like Microsoft are using their expertise in AI to “work with Adaptive Biotechnologies to develop a machine learning technology that can separate all the T-cells from a blood sample, analyze the DNA and produce a printout with information about every disease that your body is coping with”, as Peter Lee, the Corporate VP, says.



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At the same time, as the pharmaceutical industry continues to evolve and become more specialized, Contract Development and Manufacturing Organizations (CDMOs) are emerging as a crucial component in the drug development and production process. CDMOs offer a range of services, from early stage research and development to commercial scale production, allowing pharmaceutical companies to outsource specific aspects of their operations. Pierre-Alain Ruffieux, the CEO of Lonza Group, says that his company “brings the technical expertise on the manufacturing process, offers manufacturing facilities, and brings the regulatory expertise required on the quality side”. One of the global giants to join the CDMO industry in the last decades is Samsung Biologics. Its CEO, John Rim, tells us that “the aging population and the continued growth and income in technology advancements led Samsung to invest in the life sciences industry and, as a result, in 2011 founded Samsung Biologics.” Samsung’s bet seems to have paid off and it is now growing at an outstanding pace, doubling its revenue from 2021 to around \$1.9 billion. The limit of the potential to be unlocked here remains uncertain. IT has helped make supply chains more efficient. In the words of Mandar Paralkar from SAP: “Covid-19 proved that supply chain networks have to be resilient. Regulatory agencies around the world protect their

The Race to Find a Cure



STEPHANIE VEYRUN-MANETTI
| CANADA COUNTRY LEAD, SANOFI

Sanofi’s goal is to become the industry leader in immunology. We focus on finding treatments for type 2 inflammatory diseases. It is the reason we put so much effort into Dupixent, jointly developed with Regeneron.



KEVIN FITZGERALD | CSO, ALNYLAM PHARMACEUTICALS

If you have a disease where something is being produced too much, imagine an overflowing sink. You can either keep mopping the floor, which is what antibodies do, or you can turn down the faucet and let the drain catch up - what RNAi therapeutics do.



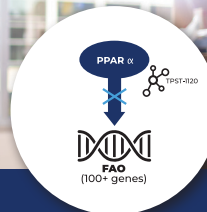
WILLIAM LEWIS | CHAIR & CEO, INSMED

Rare disease innovation has been the success story of the last two or three decades since the Orphan Drug Act aligned the FDA with the companies that were trying to develop therapies in this space.

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citizens by requiring serialization documentation of ingredients to ensure pharmaceutical products follow local regulations. All this additional paperwork increases the burden on the industry, not just when it comes to source transparency, but also for the IT systems that have to be ready to support traceability.” Jeffrey Simmons, CEO of animal health company Elanco, voices excitement and hope in view of their collaboration with the IT industry, which will help them have a wider reach: “With 69 years of history, we are at a point at which we have a network that reaches the world’s pets and animals. April 2023 will be a milestone as we roll out our new SAP system that can reach into over 100 countries. Elanco will be the bridge to the world’s animals.” From improving drug development and manufacturing, to AI and efficient supply chain, the IT industry and CDMO industries are now at the forefront of progress in the life sciences.



SEAN MCLANE
FOUNDER & CEO
ABSCI



We believe we are going to be the first company to be able to design an entire antibody on a computer from scratch using zero-shot generative AI, and bring it to the clinic. When and if we achieve this, we will be at the nexus of a pivotal point in the history.

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COURAGEOUS INNOVATION

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- Developing vaccines to provide choice in the fight against COVID-19
- Pursuing regenerative cell therapy
- Innovating a novel biologic to treat eye diseases



DAVID RICKS | CEO, ELI LILLY

Companies have periods of big losses followed by big profitability and only the largest pharma companies have a running business model. What people don't understand is that sales of today finance the cures of tomorrow. In our case, US\$ 1 out of US\$ 4 people pay for in their medicines goes into researching a disease they don't have - but might one day have.



STEPHEN BRADY | CEO, TEMPEST THERAPEUTICS

Capital markets have been volatile, and fundraising is more difficult. Companies with commercialization rights to programs have the potential optionality to combine business development and financings.



JEFF CAMBRA | CEO, SpinaFX MEDICAL

Many of the venture capitalists (VCs), private investors and family offices that we are speaking with right now are very reserved and more selective than what we have seen in the past.

The High Price of Innovation

The pharmaceutical industry has long been a source of controversy, with a public perception that is often marked by suspicion - prior to the Covid-19 pandemic, according to Gallup polls only the federal government was held in worse regard than pharma. From concerns over skyrocketing drug prices to accusations of putting profits before patients, the industry's reputation has faced numerous challenges. Admittedly, the world of pharma is not without its shortcomings; the occasional opportunism of some players to prioritize profits does exist - one needs only to remember the Sovaldi scandal in 2013, when Gilead's revolutionary cure for Hepatitis-C was priced at \$84,000 upon release, making the Californian giant double its revenue from \$11 billion to \$25 billion. It is also true that when observing some of the basic facts around the pharma industry in the U.S., one will inevitably find some conspicuous figures. First example: the U.S. spends more on prescription drugs per capita than any other country in the world, with an average cost of \$1.443 per person in 2019. Second example: prices have increased dramatically over the past decade, with some drugs seeing price hikes of over 500%.

When talking about costs and prices, it is necessary to recall some core facts that are often forgotten. Developing new drugs is a complex and expensive endeavor for pharmaceutical companies, each new development estimated at around \$2.6 billion. The process typically involves much investment risk and years of research and testing, including pre-clinical trials, clinical trials, and FDA approvals. Furthermore, the U.S. has a unique pricing ecosystem that involves insurance companies and other intermediaries (Pharmacy Benefit Managers, or PBMs) that double the price of most medications. ▶



DEBASHIS GHOSH
PRESIDENT - BTG
TATA CONSULTANCY SERVICES



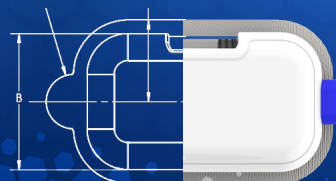
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🔴 This means that, on average, pharma companies get 50 cents out of every dollar. But before unraveling the complexity of the nation’s entire healthcare systems, some in the industry are trying to find ingenious ways to lower costs. One example is that of BeiGene, a global company aiming for this objective through their clinical trials : “up to 90% of the total cost of developing a medicine is tied up in the upfront clinical trial phase, and most clinical trials are run in wealthy countries and thus are not highly inclusive. But if more companies would run global clinical trials, as BeiGene does, we could as an industry reduce the time to enroll and conduct trials, reducing costs overall,” says CEO John Oyler.

On the flip side, Michael Laranjo, Otsuka Canada Pharmaceuticals’ CEO, talks about all the money that the life sciences save further down the line: “the truth is that some drugs help keep patients out of hospitals.” In other words, these drugs reduce the costs for both the system and patients by preventing complications. Ocugen’s CEO Shankar Musunuri, mirrors this attitude, claiming that “accessibility is also critically important” and that “it is not good enough to bring breakthrough gene therapies to market if patients cannot afford them. We need to be diligent in pricing if we want the most vulnerable populations to have access to treatment.”

It is true that much of the cornucopia of innovations North America has experienced in the past decades is linked to the existence of a potential financial reward, which in turn is linked to ‘free’ prices. Joe Panetta from Biocom California argues for this point: “The U.S. has a very different economic model in relation to healthcare than the rest of the world (...) Here we have an incentive-based,

open market system for healthcare and for the development of drugs. This has historically served as an advantage for the industry, fueling the development and commercialization of the most cutting-edge medical technologies and therapies the world has ever seen, alongside a free market providing open access.”

In other words, the industry develops because investors inject capital confident that they will enjoy returns - and these only arrive in a free market. If the market was to be more strictly regulated, costs would be lower for patients, but many fear that innovation would stagnate - a fear that might reify with the recent passing of the Inflation Reduction Act in the U.S.



MARK GODSY
FOUNDER & CEO
SHACKELFORD Pharma



Approximately one-third of the global 50 million people with epilepsy do not respond to their medication and have uncontrolled seizure activity.

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🔴 Inflation Reduction Act (IRA): The Donkey in the Room

The IRA has the potential to drastically alter the landscape of the biopharmaceutical industry. Its aim: to reduce the rate of inflation in pharmaceuticals by implementing price control measures and promoting transparency, making prescription drugs more affordable for consumers. While some proponents argue that cost control will bring much-needed relief to consumers, others - such as Mike Guerra, President and CEO of California Life Sciences, warn that the consequences could be dire: “We must be very clear on just how severe the ripple effects of the IRA, in its current form, can be. New research published by EU based Vital Transformation counts among the consequences hundreds of thousands of job losses as project funding diminishes, while VC companies are adopting a wait-and-see attitude. Also, if such drug pricing provisions had been in place, only six out of 110 therapies approved over the past decade would have made it to patients due to the limitations on research funding and partnerships.” Thus, the IRA has brought a seismic shift to the pharmaceutical industry.



DR. FRANK ROMANSKI
DIRECTOR OF MARKETING
BASF Pharma Solutions



Medicine consumption in the U.S accounts for less than 10% of the total global pharmaceutical industry by volume, while nearly 50% of the value produced is found here.

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SEAN A. MCCARTHY, D.PHIL.
 CEO & CHAIRMAN
 CytomX THERAPEUTICS, INC.



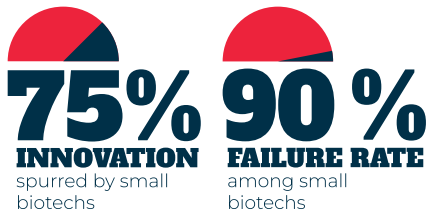
As a leader in the field of disease localized therapeutics, CytomX pushes the boundaries of technology and biology as we strive to make the biggest difference for patients.

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The Financial Turmoil of Biotech

Biotech startups were responsible for 75% of innovations in recent years; also, 90% of them fail. With the markets in turmoil and investors becoming increasingly risk-averse, securing funding has become a monumental task for young companies operating in the field. In the words of Christopher Schaber, CEO of Soligenix, “over the last two years the investment climate has been difficult due, in large part, to the broader global market conditions. As you would imagine, this has not translated well for smaller, earlier stage biotech companies, where both development risk and capital need is higher. Coupled with the length of the drug development process that can take 10-15 years in order to achieve potential success, it can make for even harsher times.” It seems like the once-plentiful streams of capital have all but dried up, leaving many promising ventures struggling to stay afloat. A more dramatic example is that of M6P, a small startup with remarkable science that holds a strong promise to treat lysosomal storage disorders (LSDs) through enzyme replacement therapies. Hung Do and Cuong Do, M6P’s leadership team, tell us that “unfortunately, at the moment, the entire biotech investing market, especially for preclinical companies, is frozen, so we are now relying on existing shareholders to keep the development going.” Despite hardship, the biotech community has remained steadfast in their pursuit of medical innovation. Companies like Soligenix and M6P continue working against the tide, with a rich pipeline that promises much.

Now more than ever, it seems that collaborations and acquisitions between big pharma and biotech are crucial in keeping the wheels spinning. Merck’s spin-off Organon is poised to change the landscape of women’s health. Its CEO, Kevin Ali, tells us that despite their being “a young company with a 2021 IPO” they “have completed 8 deals in the past 18 months, and are rapidly growing.”



MARK GOLDBERG
 | MD, CHAIRMAN & CEO, ALLUCENT

Small and mid-sized biotech are responsible for as much as 75% to 80% of the new developments in our industry. The programs they’re working on are often so new that new regulatory pathways need to be explored.



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They are offering products such as the Jada device which addresses postpartum hemorrhage or abnormal bleeding in childbirth at a “90% plus efficacy rate and acts in under three minutes.” This is a big leap in an industry that has been underfunded and deprioritized in the past, treated as a niche, but as Duchesnay CEO Eric Gervais points out, “the four billion women in the world are not a niche percentage.”

Innovation hubs such as the Pennsylvania Biotechnology Center also bear good news, showing that the industry still stands strong in Pennsylvania, where “the last six years generated more than \$7.3 billion worth of economic impact.” This is a testament to the resilience and innovation of the industry, as well as the vital role that biotechnology plays - and will play - in our society. Sekar Kathiresan is the CEO of Massachusetts based Verve Therapeutics, a company that fights heart disease through fascinating novel techniques such as gene editing, claims his company “has raised about \$800 million in capital across three private rounds and two public financings. Since inception, Verve has spent about \$250 million, so we have a significant amount (about \$550 million) still left at our disposal to continue the development of this medicine.



CHRISTIAN HENRY
CEO
PacBio



PacBio intends to create a paradigm shift demonstrating that our technologies deliver better resolution, with competitive economics, at scale.

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”Perhaps the clearest sign that there is trust in the sector comes from companies such as EY, whose “pharma sector is currently the fastest growing sector at EY globally,” as Pamela Spence, Global Head Sciences leader, assures. Despite challenges such as funding cuts and regulatory hurdles, biotechnology companies and the life sciences in general continue to make significant strides, bringing cutting-edge technologies to market and making a real difference in the lives of patients.

From Tragedy to Action: the Power of Personal Stories

On interviewing many of the leaders in North America’s life sciences industry, one thing was particularly unexpected: many of the people we talked with were driven primarily by personal stories. Experiences of illness and loss serve as a powerful impetus for many. In our encounters, this was most commonly observed in the rare diseases sector.



WALTER KORZ | COO, WEX PHARMACEUTICALS
Opioids have been around since the early 1800s, and they are still what we always use for severe pain for patients post-surgery. They are useful and necessary, but not in all painful conditions.

John F. Crowley, Executive Chairman of Amicus Therapeutics, is a father of two children born with Pompe disease, which is a rare genetic disorder. Faced with the devastating reality that there was no cure for his children’s condition, Crowley refused to give up hope. He quit his corporate job and, with unwavering determination, set out on a mission to find a cure. Soon after entering the world of biopharma, Crowley founded Amicus. Today, his work at Amicus has had a direct impact on his children’s lives and many others with Pompe disease. “At the end of the day” Crowley tells us “biotechnology is just a great big word that means hope.”

A NEW APPROACH TO PAIN MANAGEMENT

WEX Pharmaceuticals Inc. is developing a late-stage non-opioid analgesic, **Halneuron®**, which has been tested in over 700 people and 15 clinical trials. Halneuron is preparing for pivotal Phase III clinical trials for chemotherapy-induced neuropathic pain and is also being tested for other pain indications.



www.wexpharma.com

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For PTC Therapeutics, our 25-year history has centered on bold, transformative science that brings more moments to patients and their families living with rare diseases.

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TRANSLATING SCIENCE Transforming Lives

Extending life's moments for
people living with rare diseases



ERIC GREEN

PRESIDENT, CEO AND CHAIR OF THE BOARD
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This year we celebrate our 100-year anniversary, a milestone that few companies achieve. Most importantly, our purpose has remained the same: Improving patients' lives across the globe.

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years

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On a different, deeply personal note, Traverre's CEO Eric Dube tells us "Coming out as gay at the worst point during the AIDS epidemic in the '90s, I saw how little society, the government, and healthcare providers valued the lives of the men who were dying (...) In my career, I use my position to advocate for the health of all communities: to give a voice to patients who might feel like they lack access to the necessary medical care." Dube went on to found Traverre, which specializes in rare diseases. Their products help thousands of Americans with rare diseases live better lives. Another noteworthy example, this time outside the field of rare diseases, is Branislav Vajdic, CEO and Founder of HeartBeam. Vajdic tells us about his main motivation: "My father was a physician, and one day at home he started feeling some tightness in his chest. He took this for indigestion, but it turned out to be a fatal heart attack - potentially one that could have been non-lethal, had it been addressed in time. This experience shook me, and harnessing all my knowledge of technology I asked the question: is there any technology out there that can help detect a heart attack?" Since then, Vajdic has devoted his efforts to developing a new generation of cardiovascular devices, the ingenious AimiGo, which has the potential to dramatically reduce the risk of a heart attack fatality by monitoring heart function with a credit-card size device.

From a debilitating medical condition to stories of uphill battles and shocking experiences where everything seems to crumble, these experiences serve as a driving force behind the development of new and innovative solutions, aiming to prevent similar suffering in the future. Reflecting upon the testimonies of these leaders, we are reminded that progress is not limited to scientific advancement, but also emanates from the spirit of those fighting with unwavering resolve.

As we embark on this new era of life sciences, both hope and challenges lie ahead - reminiscent of every revolution in human history. At what price, in what context, and what will be the impact of the medical breakthroughs discussed in this report on our lives? Only time can tell. We navigate a complex landscape where different forces cohabit, and it sometimes seems hard to see through it at all - especially when talking about an industry that raises many passionate and often opposing opinions. Undeniable are the stories, innovations and efforts of those who believe in the industry and improve the lives of patients every day.



JOHN RIM
PRESIDENT & CEO
SAMSUNG BIOLOGICS



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