



## Accelerating R&D productivity and industry collaboration

### Driving productivity, a need for speed

Currently, many life sciences companies are showing a 'need for speed' as their focus is on driving research and development (R&D) productivity. New processes adopted to expedite vaccines and therapeutic products to tackle COVID-19 are now being applied to other drugs. In 2022, pressures are expected to be on optimizing processes to fundamentally change the drug development paradigm.<sup>1</sup>

“

Collaboration and digitalization have played fundamental roles in bringing COVID vaccines and therapies to market at an unprecedented rate, saving an estimated 750,000 lives in the United States and Europe alone. As an industry, we must bring the same speed and sense of urgency to all our efforts.

”

*Paul Hudson, CEO, Sanofi*<sup>2</sup>



## Impact on timelines

While some worried that life sciences companies would see a negative impact on timelines during the pandemic, others were more bullish about the degree of change life sciences companies were going to embrace and mobilize to accelerate timelines. At large, timelines didn't change.

What are typical timelines? Research conducted by Cowen analyzed the performance of 11 leading pharmaceutical companies over more than 15,000 clinical trials across five categories. The time period analyzed was 2012-2021, unless otherwise noted.<sup>3</sup>

- Pipeline pace: Average time to reach approval, almost 5 years
- Recruiting speed: Average time to enroll patients in trials, almost 19 months
- R&D efficiency (2016-2021): Average R&D spend per active trial, US\$35 million, US\$66 million per active Phase III trial (may reflect a proclivity to in-license late stage assets)
- Trial push-outs: Average percentage of trials not delayed, 52%
- Delay duration: Average change in primary completion rate, 57-day delay<sup>4</sup>

If we look at the results for recruiting speed, (see figure 1)<sup>5</sup> for example, the data show how timelines for recruiting vary by company and therapeutic area. While it may take only about 4 months to enroll healthy participants, it may take almost 30 months to enroll participants for studies concerning ulcers and the gastrointestinal tract.<sup>6</sup>

Figure 1: Trial recruiting speed by company and therapeutic area, 2012-2021

Weighted average enrollment time in months by company (left) and by therapeutic area (right)



Source: "R&D Pentathlon: Which Pharma's R&D is faster, higher, stronger —ahead of the curve," Cowen, 26 July 2021.



Overall, the leading companies averaged about 19 months to enroll patients, and the best recruiting speed—significantly leading the rest—was Novo Nordisk, with a phase-weighted average of 9.6 months. However, the company's absence in oncology trials was a tailwind. The average oncology trial took 27.6 months to enroll, compared to non-oncology trials at 14.9 months. Second-place GlaxoSmithKline (GSK) and third-place Eli Lilly also had limited exposure to oncology.<sup>7</sup>

Pharma R&D recruiting timelines were not slowed down by the pandemic. However, clinical trial starts actually increased and are back on track.<sup>8</sup> Where we did see shortened timelines, companies are keen to replicate, if possible.

Deloitte's analysis of R&D cycle times for 15 leading pharmaceutical companies shows that, prepandemic, average cycle time was 6.64 years in 2019, followed by an increase to 7.14 years in 2020, a 7-year high. A slight improvement came in 2021—6.9 years—a decrease driven by the expedited completion of studies for COVID-19 therapies and vaccines.<sup>9</sup>

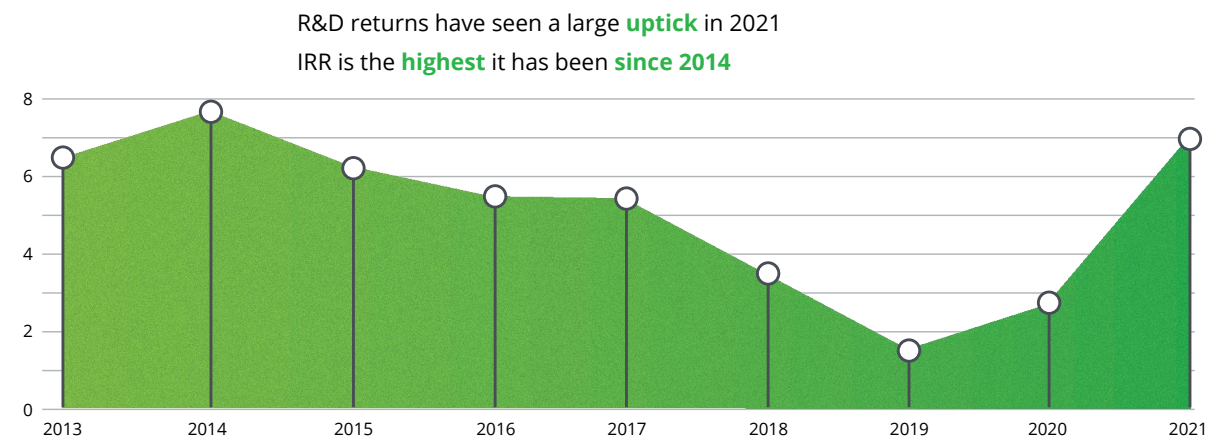
Some accelerated paths for improving productivity include focusing on continued digital transformation, quality and safety, and working in tandem and across geographies with regulators for earlier approvals.<sup>10</sup>

## Return on pharmaceutical innovation

Deloitte has been tracking return on pharmaceutical innovation since 2010. Up until 2020, companies we tracked experienced a decade-long decline in R&D productivity. But in 2021, Deloitte's analysis of 15 large pharmaceutical companies shows a significant uptick for the cohort's internal rate of return (IRR)—7%, up from 2.7% in 2020. COVID-19 related assets buoyed by emergency approvals played a significant role. Excluding those assets, the projected IRR is still higher, at 3.2% (see figure 2).<sup>11</sup>

Figure 2: Uptick in R&D returns for biopharma cohort for 2021

15 biopharma companies were analyzed by Deloitte



Source: Nurturing growth, Measuring the return from, pharmaceutical innovation 2021, Deloitte Centre for Health Solutions UK, January 2022.





Pharmaceutical companies expect to advance scientific breakthroughs and manufacture innovative products in an effort to fulfill unmet needs and stay ahead of the competition. In 2021, the average cost to develop an asset for this group, including the cost of failure, decreased US\$70 million from 2020, to approximately US\$2 billion. This decrease is mainly due to the increase in the number of assets in the late-stage pipeline for the companies analyzed. In 2021, the group had a total number of 242 late-stage assets, an increase from 210 in 2020.<sup>12</sup>

“ We can't revert to our old ways. ”

*Paul Hudson, CEO, Sanofi*<sup>13</sup>

### Tracking R&D spend

Drug Discovery & Development analyzed the R&D spend for leading pharmaceutical companies' annual reports. Spending for the top 15 companies ranged from almost US\$4 billion to more than US\$13.5 billion for the year (see figure 3).<sup>14</sup> For R&D spend as a percentage of revenue, Incyte led the industry with an R&D spend of US\$2.2 billion that constituted a whopping 83.10% of its revenue. Regeneron Pharmaceuticals spent almost US\$2.4 billion on R&D, or 32.19% of revenue, to claim the number two spot.<sup>15</sup>

When allocating R&D spend for an asset, a pharmaceutical company is often influenced by:

- Anticipated lifetime global revenues from the new drug
- Expected costs to develop the drug
- Policies and programs influencing supply of and demand for prescription drugs<sup>16</sup>
- Pressure to innovate<sup>17</sup>

Pharmaceutical companies face greater pressure to innovate because of the time-limited patent protection of their name-brand drugs. When a patent expires, companies expect to take a big hit to sales from generics and biosimilars and plan accordingly.<sup>18</sup>

Figure 3: Pharma companies' total R&D spend in 2020

	Company	USD for 2020
1	<b>Merck</b>	\$13,558,000,000
2	<b>Roche Pharmaceuticals</b> (division of Roche Group)	\$12,164,234,743
3	<b>Bristol Myers Squibb</b>	\$11,143,000,000
4	<b>Janssen</b> (Johnson & Johnson's pharmaceutical segment)	\$9,563,000,000
5	<b>Pfizer</b>	\$9,405,000,000
6	<b>Novartis</b>	\$8,980,000,000
7	<b>AbbVie</b>	\$6,557,000,000
8	<b>GlaxoSmithKline</b>	\$6,509,126,400
9	<b>Sanofi</b>	\$6,303,060,000
10	<b>Eli Lilly</b>	\$6,086,000,000
11	<b>AstraZeneca</b>	\$5,991,000,000
12	<b>Gilead Sciences</b>	\$5,039,000,000
13	<b>Takeda Pharmaceutical</b>	\$4,611,350,440
14	<b>Amgen</b>	\$4,207,000,000
15	<b>Biogen</b>	\$3,990,900,000

Source: Drug & Discovery 2021



## Establishing new norms

### Revamping the clinical trial model

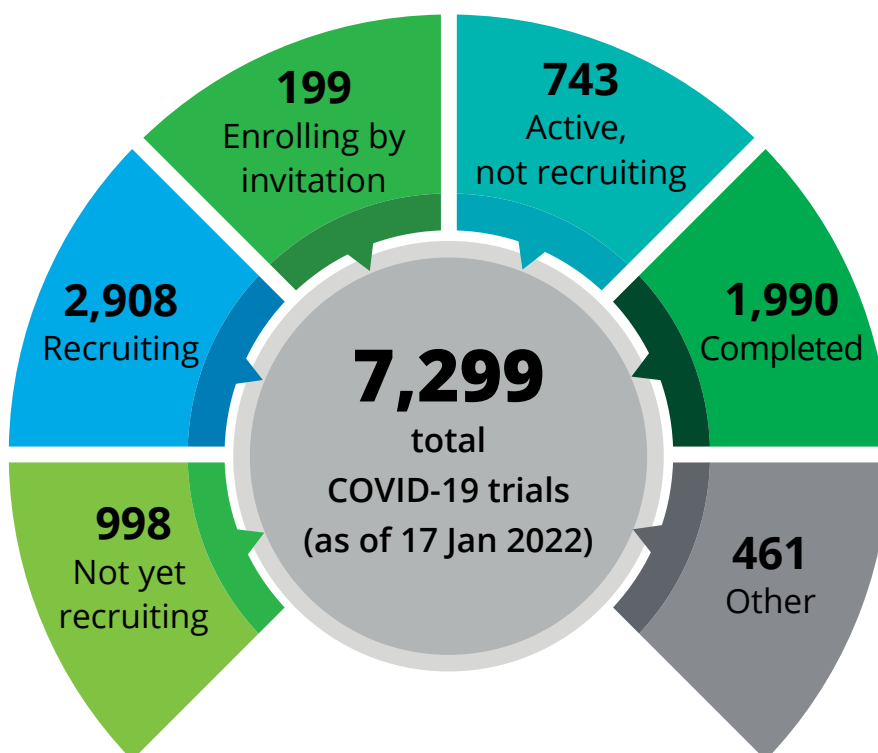
Changes brought about as a result of the COVID-19 pandemic are shaping a new era in clinical trials. Restrictive norms and outdated processes are falling away. With digital and virtual tools, constraints such as geography and set 'business hours' are no longer barriers to participation. Researchers are finding new ways to bring more people into trials through new models that are adaptive, decentralized, and hybrid. Participants expect more personalized care and real-time access, no matter where they are.<sup>20</sup>

Remote monitoring and remote visits were top strategies for keeping clinical trials open during the pandemic.<sup>21</sup> Research shows that there were more study starts across the board by the end of 2021 than ever before, with an increase of almost 18% from 2020 to 2021, including more non-COVID trials. Oncology saw the biggest increase at almost 1,300 trial initiations in 2021, a 23% increase.<sup>22</sup> Two years after the pandemic started, more than 7,000 clinical trials had been initiated related to COVID-19 in the NIH Clinical Trials database (see figure 4).<sup>23</sup>

### Investing in the research lab of the future

Accelerated advancements in drug discovery and delivery are expected to fuel industry growth and demand for lab space in the near term.<sup>19</sup> In the R&D lab of the future, an interconnected ecosystem of data, platforms, instruments, and advanced analytical tools supports scientists across teams and geographies to rapidly discover breakthrough therapies. In 2021, Deloitte surveyed 150 leaders across the value chain from large biopharma companies (revenue of US\$1 billion and above) across the United States, Europe, and Asia. Survey respondents say that their organizations are currently prioritizing investments in AI (81%) and cloud (71%). A much smaller percentage believes that over the next five years their organizations are likely to invest in AR/VR (19%) and IoT (24%), both of which are essential for the lab of the future.

Figure 4. Total number of clinical trials related to COVID-19 vaccines and treatments as of January 17, 2022



Source: NIH Clinical Trials



Many hospitals and research institutions shifted from in-person clinical trials to home-based reporting and the clinical trial process was rapidly digitalized with tools such as telemedicine, sensor-based technologies, and wearable medical devices. Many trial participants were able to receive direct-to-patient shipment of investigational products and trial supplies, and remote consent was enabled via mobile—further modernizing the clinical trial process.<sup>24</sup>

### ***Adapting trials to patient response***

Adaptive clinical trials are using the potential of AI to discover the best possible treatments. An adaptive trial can be modified according to a patient's response. For example, if a drug is not working, it might be pulled, and another treatment readily started in its place.<sup>25</sup> Researchers used adaptive trials to discover and compare potential COVID-19 treatments during the pandemic. The World Health Organization and groups like the Bill & Melinda Gates Foundation are showing increasing support for adaptive trial designs, particularly as a way to evaluate therapies during epidemics.<sup>26</sup>

“ The pace of change in trial design and execution will continue to accelerate through adaptive trial design, which allows monitoring of incoming data and modifying the trial protocol based on interim review. Amgen has implemented these tools in clinical programs in inflammation, cardiovascular disease, and others disease areas. ”

*Elliott M. Levy, MD, Senior Vice President, R&D Strategy and Operations, Amgen* <sup>27</sup>

### ***Reducing patient burden through decentralized trials***

New types of trials are already proving to be more productive. In particular, research shows that decentralized trials (DCTs) that rely on remote strategies are showing return on investment and are here to stay.<sup>28</sup> Digitization is reducing patient burden and reporting subjectivity, while increasing outcome accuracy.<sup>29</sup> In developing MyPath for Clinical, Deloitte found that an end-to-end digital platform for decentralized trials was a more streamlined way to address many patient challenges—such as traveling to sites, confusion about treatment protocols, and access to medical teams in real time.<sup>30</sup>

In a fully decentralized clinical trial, patient recruitment, delivery of trial medication and supplies, and acquisition of trial outcomes data is enabled through virtual and real-time tools—essentially eliminating the need for in-person contact between the study team and the trial participant.<sup>31</sup>





### Tufts' study finds 5x to 14x financial return on decentralized trials investment

A recent study from the **Tufts Center for the Study of Drug Development** compared published benchmarks on trial cycle times and costs with data from more than 150 decentralized trials conducted by software firm **Medable**. The study reports that, on average, decentralized clinical trials (DCTs) are associated with reduced clinical trial timelines, recruitment, and higher retention rates. For example, decentralized phase 2 studies were completed 1 to 3 months faster than traditional trials, a net benefit up to five times greater than the upfront investment required; phase 3 was 14 times greater.<sup>32</sup>

### Improving access to trials and technology through hybrid trials

Decentralized trials and remote monitoring may address convenience and diverse populations in distant locations, but underserved and minority populations are still likely to experience disparities. For example, access to broadband, caregivers, and home health care, may present a challenge.<sup>33</sup>

Not all trials can be fully decentralized. Hybrid trials, that also reach patients through the community where they live, are another growing solution.<sup>34</sup> Researchers should be aware of the logistical difficulties that some necessary in-person provider interactions and laboratory tests may pose for some participants. For example, some may face travel constraints (e.g., access and cost), nonacceptance of job absences for study activities, and mobility challenges due to medical comorbidities.<sup>35</sup>

### CVS Health expands access to clinical trials through retail locations

**CVS Health Clinical Trial Services** was initially launched in response to the pandemic with the goal of providing greater access to clinical trials across the communities it serves. Collaborating with the pharmaceutical industry, CVS Health helped facilitate clinical trials for investigational COVID-19 vaccines and treatments. Using a specially designed digital model and screening protocols, the company engaged more than 300,000 volunteers for COVID-19 vaccine trial consideration, connecting them to studies close to where they live.<sup>36</sup>

By creating a more efficient, convenient experience to improve participant retention and research effectiveness, CVS Health believes its new clinical trial experience benefits participants, health care providers, clinical research organizations, and study sponsors. The business is initially focused on scaling three core capabilities: precision patient recruitment, clinical trial delivery, and real-world evidence generation and studies.<sup>37</sup>

Localizing in-person visits and providing digital tools to make it easier for patients to participate in their own communities may be all that is needed.

By making the clinical trial process more convenient for enrollment and participation, industry leaders stand to gain better research results, fewer failed trials, and more trust from physicians and patients. More accessible trials for diverse populations may provide deeper insights, enabling more tailored drug therapies to be developed and more productive R&D.<sup>38</sup>

### Defining goals for clinical trial diversity

The Pharmaceutical Research and Manufacturers of America (PhRMA) is an industry organization committed to enhancing diversity in clinical trial participation. In last year's Life Sciences Outlook, we introduced PhRMA's industry-wide "[Principles on conduct of clinical trials and communication of clinical trial results](#)".<sup>39</sup>

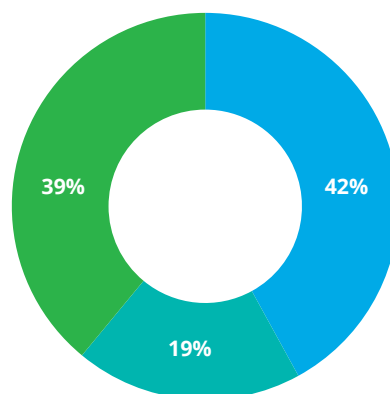


In 2021, PhRMA and the Deloitte US Center for Health Solutions conducted [extensive research on clinical trial diversity](#), including a survey with 31 PhRMA's member companies and a workshop with more than 500 stakeholders from more than 150 organizations. The survey of PhRMA members showed 61% of respondents have defined goals and objectives to enhance clinical trial diversity, and all respondents have or are planning to address trial access issues (see figure 5).<sup>40</sup>

Figure 5: PhRMA member companies survey on clinical trial diversity

**Percentage of respondents with defined goals and objectives for increasing clinical trial diversity**

- We have defined goals and objectives, identified best practices, and begun to incorporate these across some of our trials
- We have defined goals and objectives and working to identify best practices
- We are working on defining our goals and objectives



Note: N=31 PhRMA member companies

**All respondents have or are planning to address trial access issues and are considering the needs of diverse populations in clinical trial design**

We are taking specific measures to address trial access issues ( e.g., transportation costs, event scheduling, remote/decentralized data collection, patient apps and data access, etc.)



We are considering the needs of diverse populations in clinical trial design (e.g., taking a patient-centric approach to protocol design and incorporating patient input)



We are identifying sites where diverse patients may be located, identifying health care providers that treat underserved or underrepresented populations, and collaborating with investigators to address the goals of enrolling a diverse population



We are enhancing education on the role of clinical trials throughout the medical community



We are increasing clinical trial awareness and diversity by improving individual health literacy and community outreach



We are enhancing information about diversity and inclusion in clinical trial participation (e.g., developing and maintaining policies and procedures, making these publicly available)



We are using real-world data to enhance information on diverse populations beyond product approval



We are enhancing diversity among clinical investigators



We are broadening eligibility criteria to increase diversity in enrollment when scientifically and clinically appropriate



Note: N=31 PhRMA member companies

■ Yes ■ No

Source: "Enhancing clinical trial diversity," Deloitte Center for Health Solutions, 2021.



Most surveyed PhRMA member companies identified areas to address internally to enhance clinical trial diversity—such as legacy processes and systems, data on demographics of disease by race/ethnicity, and protocol design flexibility. Five key strategies were identified for enhancing clinical trial diversity and are discussed in the **ESG section** of this report.

## Evolution of real-world evidence

### *Amount and types of data rapidly accelerating*

Life sciences companies strive for consistent, regulatory-quality clinical trial data to prove the effectiveness of treatments, and researchers are expected to gather, analyze, and curate many streams of structured and unstructured data. The amount of data being collected by digital health technologies—telemedicine, mobile devices, wearables, and other sensor-based technologies—is rapidly accelerating due to decentralized trials and remote monitoring.<sup>41</sup>

In addition to clinical data, genomic information and improvements in technology—like AI and quantum computing—are evolving the way life science organizations approach drug discovery and development. Real-world data (RWD) and real-world evidence (RWE) collected today have the potential to better inform clinical trial design/execution and deliver insights never before thought possible. In Deloitte's annual survey of C-suite biopharma leaders, 100% identified RWE as strategically important.<sup>42</sup>

At the end of 2021, the US Food and Drug Administration (US FDA) issued draft guidance for using digital health technologies to acquire data remotely from participants in clinical trials. In addition to sponsors and investigators, developers and manufacturers have the opportunity to benefit by reviewing this guidance.<sup>44</sup>

### *Selecting digital tools for better clinical trials*

When selecting digital and virtual tools for clinical investigations, sponsors should ensure they are “fit-for-purpose” or sufficient to support their use and interpretability in the study. Data transmission with manufacturers and end-user licensing agreements should be designed to secure data collected and transmitted.<sup>45</sup>

Getting better data from devices in clinical trials:

- When validating instruments for trials, collect sufficient data to understand how each tool really works. Some tools may not transfer to a virtual platform.<sup>46</sup>
- Ensure that digital tools have user-friendly interfaces for supervisors and patients.<sup>47</sup>
- Wearables help take subjectiveness out of the data, but wearables are not always accurate. Always question data, and how you are interpreting the data.<sup>48</sup>
- Check that tools are measuring what you need them to measure. Is the data being collected clinically relevant and relevant to the patient?<sup>49</sup>

Teams designing trials should have a clinical expert involved, not just statisticians. These experts have the ability to bring clinical analysis a priori and to look at outliers for clinical relevance. Value is expected to come from human-enabled AI—expert clinical knowledge coupled with AI and machine learning (ML) analysis.<sup>50</sup>

### **Verana Health: Building a vast database of RWE for clinical R&D**

**Verana Health** is building an extensive database of real-world clinical evidence curated from organizations like the American Academy of Ophthalmology, the American Academy of Neurology, and the American Urological Association. Life sciences companies and health care providers can mine the platform for insights from the electronic health record (EHR) systems of more than 20,000 health care providers. Their population health analytics tool, VeraQ, uses AI to automatically sift through those data, helping drug and device developers with their research and speeding up clinical trial recruitment. The startup has recently garnered support and funding from Johnson & Johnson, Novo Nordisk, and Merck.<sup>43</sup>



Through AI & ML, life sciences companies have also been able to select investigators and countries for their clinical trials as well as predict their performance.<sup>51</sup>

### ***Lessons from the COVID-19 experience***

In 2022, biopharma companies are looking to apply some of the more successful COVID-19 development measures to plan, design, and execute studies more efficiently by:

- Enabling at-risk development for high-priority programs, allowing them to bypass certain stage-gates
- Expanding collaborative dialogue with regulators, using data-sharing infrastructure and harmonizing across geographies
- Limiting the number of relevant endpoints, to streamline trial protocol design
- Enabling the rapid assessment and development of therapies with master protocols and adaptive trial design
- Accelerating the use of digital technologies, for conducting decentralized and hybrid studies, optimizing site selection, recruiting diverse study populations, and remotely collecting data and monitoring patients<sup>52</sup>

During the pandemic, it became both difficult and potentially dangerous for some clinical trial patients to go to the hospital. According to Badhri Srinivasan, head of global development operations for Novartis, his teams worked to set up solutions such as in-home nursing programs or direct to patient shipments of their study medication. "These are options that patients may prefer, regardless of restrictions, and many trial sites have now expanded their capabilities to accommodate these services. Therefore, we need to prioritize building assessment of decentralized clinical trial elements, such as home nursing," he says.<sup>53</sup>

## **New therapeutic modalities are the future of R&D productivity**

### ***Highly anticipated biopharma research***

The science of therapeutics is maturing with new and compelling modalities. Scientists are now focusing on diseases that were once thought intractable.<sup>54</sup> Technology and science are converging, and the modality landscape is evolving—from sporadic disease, small molecules, biologics, and protein targets to genetic disorders, Antisense Oligonucleotides, siRNA, gene therapy, and nucleic acid targets, according to Anabella Villalobos, PhD, senior vice-president at Biogen.<sup>55</sup>

Villalobos says that protein degraders are an emerging therapeutic modality showing encouraging results.<sup>56</sup> Targeted protein degradation (TPD) tackles disease-causing proteins that have historically been highly challenging to target with conventional small molecules. In the future, this modality is expected to expand the use of ubiquitin ligases to enable precision medicine and move beyond oncology.<sup>57</sup>

Dr. Jay Bradner oversees 5600 scientists and 325 discovery programs over 8 disease areas as president of Novartis Institutes for Biomedical Research (NIBR).<sup>58</sup> He says that it is ironic that returns are diminishing when our understanding of disease has never been better and more granular. He believes R&D productivity can grow thanks to new modalities.

“ My least favorite term in our field is ‘undruggable target.’ ”

*Dr. Jay Bradner, President, Novartis Institutes for BioMedical Research, Novartis*

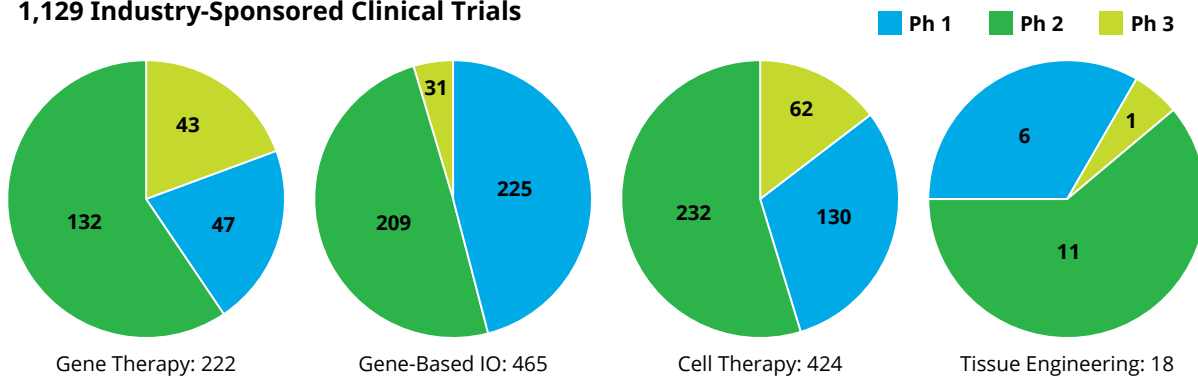


### What's next for cell & gene therapies?

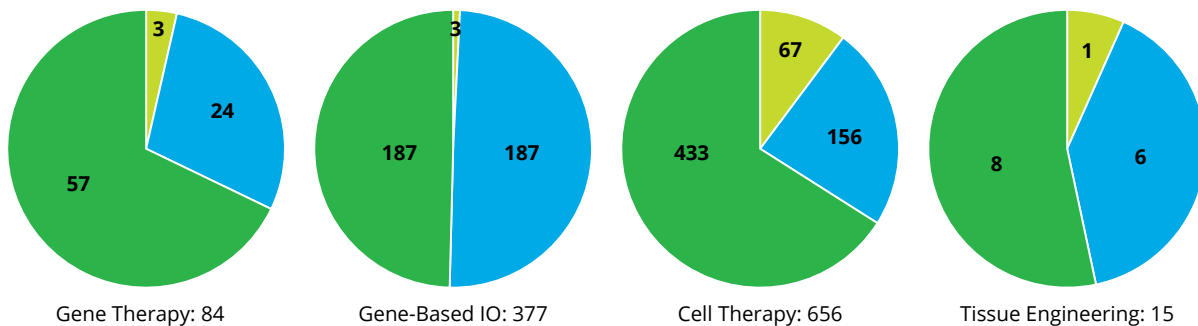
Globally, cell and gene therapies (CGTs) are transforming how humans treat genetic and intractable diseases as well as altering the entire pharmaceutical ecosystem.<sup>59</sup> As of Q1 2021, there were 2,261 ongoing global cell, gene, and tissue clinical trials—1,129 were industry-sponsored and 1,132 were academic/government-sponsored trials (see figure 6). In 2022, there will be more opportunities to expand cell and gene therapies, especially in rare disease, and more interest in allogeneic therapies.<sup>60</sup>

Figure 6: Cell and Gene clinical trials in development, March 2021

#### 1,129 Industry-Sponsored Clinical Trials



#### 1,132 Academic & Government Sponsored Trials



Data as of March 2021 by Global Data

Source: ARM, 10 January, 2022

“

We need to listen because ideas come from anywhere in the organization. We just need to evaluate and see what we can fund. Whether we do that internally or externally is another question. But we need to make sure that we listen to the project teams who are driving the science.

”

*Anabella Villalobos, PhD, Senior Vice-President, Biotherapeutics and Medicinal Sciences, Biogen*

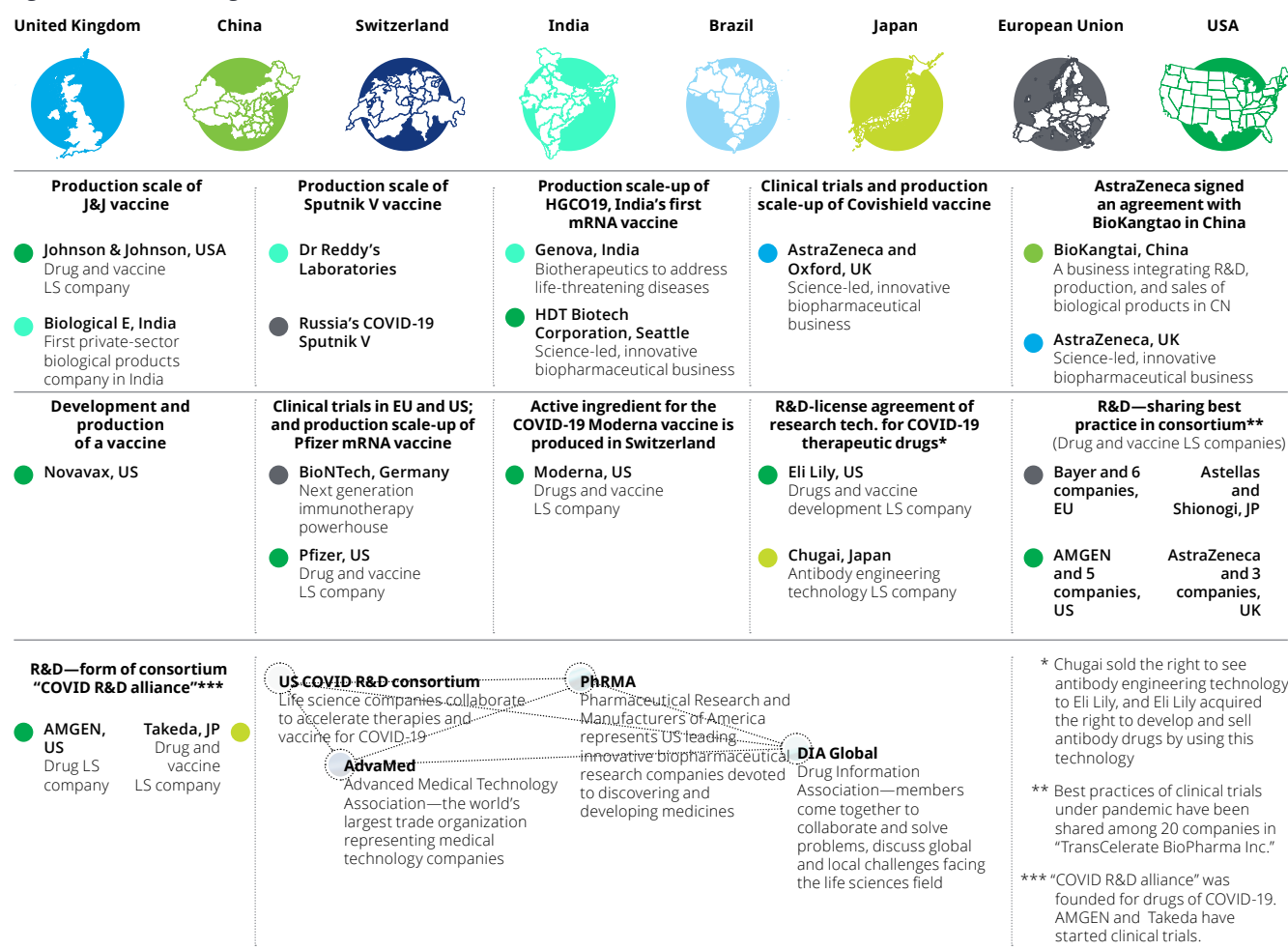


## Unprecedented collaboration

### Growing global ecosystem

During the pandemic, many life sciences companies that had been competitors came together to solve scientific problems to address the urgent need for treatments, vaccines, diagnostics, and medical devices (see figure 7).<sup>61</sup> Companies, including those in other industries, also joined the effort to help fill raw materials shortages, digitalize more of the drug development process, and enhance manufacturing at high capacities at different locations worldwide.<sup>62</sup>

Figure 7: Life sciences' global collaborations



Source: "Never the same again, How COVID-19 created seismic change in life sciences regulations," Deloitte, 2021.

Currently, life sciences companies are partnering to develop digital medicine products, playing to their strengths in regulatory science and market access, while leveraging partner expertise in software development.<sup>63</sup> Digital product development is an iterative process, and pharmaceutical companies are expected to demonstrate a willingness to be adaptive and nimble.<sup>64</sup> Most technology companies are interested in a meaningful commercial relationship and not just running pilots.<sup>65</sup>



### ***New partnerships filling the demand for digital therapeutics***

Last year, we saw pharma companies forming partnerships with digital therapeutics startups.

- Boehringer Ingelheim partnered with Click Therapeutics to develop a digital therapeutic for patients with schizophrenia
- Sanofi partnered with Happify Health to build a digital therapeutic to help multiple sclerosis patients manage their mental health<sup>66</sup>

In 2022, as the demand for digital therapeutics grows across the ecosystem, new business models will likely emerge beyond the traditional commercialization approaches. We expect to see a shift in favor of insourcing some of this technology as life sciences companies gain more experience in the field.<sup>67</sup>

### ***Mergers & Acquisitions (M&A) for R&D pipelines***

COVID-19 negatively impacted the degree of M&A in 2020, with a slight rebound in 2021—but overall activity remained subdued. There were fewer, smaller deals, and we saw companies shifting their portfolio toward rare diseases through their smaller-scale M&A activity.<sup>68</sup>

Pfizer was particularly active in 2021. Flush with cash from its hugely successful COVID-19 vaccine with BioNTech, Pfizer invested in other pipeline-boosting deals, including a US\$2.4 billion licensing deal for an Arvinas cancer drug. The success of COVID-19 vaccines from Pfizer/BioNTech and Moderna put mRNA platforms into the spotlight. Sanofi paid US\$3.2 billion for Translate Bio and its mRNA platform for expressing proteins.<sup>69</sup>

In our outlook, we expect continued focus on bolt-on acquisitions aimed at bulking up R&D pipelines.<sup>70</sup> With smaller firms, talent, tools, and intellectual property may be ported to the parent, adding value in many ways.<sup>71</sup> When smaller bolt-on acquisitions dominate, the number of deals may be high,<sup>72</sup> and deal-making activity is expected to pick up this year.

As smaller biotechnology firms and larger pharmaceutical companies position themselves for opportunities to acquire assets, we expect to see many more types of collaborative relationships maximizing new technologies. Pfizer kicked off 2022 with a US\$300 million multi-target research collaboration with gene-editing firm Beam Therapeutics. The deal makes Beam eligible to receive future milestone payments of up to US\$1.05 billion—a potential total consideration of up to US\$1.35 billion. Pfizer looks to leverage Beam's proprietary in vivo delivery technologies, which use mRNA and lipid nanoparticles (LNP) to deliver base editors to target organs.<sup>73</sup>

### ***More investments in cell & gene therapies***

Mergers and acquisitions dominated the funding for cell and gene therapies in 2021, and it appears the trend will continue. CGT represents roughly a third of all private investment in life sciences.<sup>74</sup> Total funding from all sources for cell and gene companies in 2021 exceeded US\$70.8 billion last year, including:



- IPO and FPO Deals
- SPAC Deals
- Venture Capital and Private Funding
- Strategic Investments
- Mergers & Acquisitions (M&A)
- Technology and Research Collaborations
- Licensing Deals
- Manufacturing and Supply Chain Deals
- Public Outreach, Consumer Education, & Philanthropy
- Distribution & Co-marketing Deals
- Restructurings, Joint Ventures, Terminations and Spinoffs
- Other Types of Deals Impacting Cell and Gene Therapy<sup>75</sup>



More than 50 partnership deals were executed between pharma and promising CGT companies before the first therapy was even approved in 2017.<sup>76</sup> Tools such as CRISPR and next-generation sequencing have lowered the barriers to entry,<sup>77</sup> and early approvals are paving the way for new entrants in this nascent market, where CGT represents only 7% of the approved 340 biologics.<sup>78</sup> A record number of regulatory decisions are upcoming in 2022-2023 (see figure 8).<sup>79</sup>

Figure 8: Upcoming cell & gene therapy regulatory decisions, 2022-2023

United States	<b>Gamida Cell</b> Blood Cancer + HSCT	<b>Atara Bio</b> EBV + PTLD	<b>CRISPR/Vertex</b> Sickle Cell & Beta Thal
	<b>Iovance</b> Melanoma	<b>Krystal Bio</b> Epidermolysis Bullosa	<b>CARsgen</b> Multiple Myeloma
	<b>BioMarin</b> Hemophilia A	<b>uniQure/CSL</b> Hemophilia B	<b>Orchard</b> MLD
	<b>PTC Therapeutics</b> AADC Deficiency	<b>Bluebird bio</b> Beta thalassemia	<b>Adaptimmune</b> Synovial Sarcoma
	<b>Janssen/Legend</b> Multiple Myeloma	<b>Bluebird bio</b> Cerebral ALD	<b>Bluebird bio</b> Sickle Cell Disease
2022			2023
Europe	<b>Janssen/Legend</b> Multiple Myeloma	<b>BioMarin</b> Hemophilia A	<b>Orchard</b> Wiskott-Aldrich
	<b>PTC Therapeutics</b> AADC Deficiency	<b>Atara Bio</b> EBV + PTLD	
	<b>GenSight Bio</b> LHON	<b>uniQure/CSL</b> Hemophilia B	

 BLA/MAA submitted  
 BLA/MAA planned

Source: ARM, 10 January, 2022

A significant amount of future success hinges on whether approved treatments are allogeneic or autologous. Since autologous treatments are based on a patient's own cells and made to order, they represent more complexity and cost in treatment planning, manufacturing, and supply chain. Allogeneic treatments are based on cells from a donor, fitting more into the traditional model of distribution.<sup>80</sup>

Currently, as the number of therapies is small and the volume of patients is large, payers have been open to innovative approaches to reimbursements, including annuity and installment-based payments and outcomes-based reimbursement. Experts say it is unclear how these approaches might scale as the numbers rise.<sup>81</sup>

However, the future promise of cell and gene therapies is making companies with strong cell or gene therapy pipelines and the most promising technologies, researchers, and intellectual property attractive targets.<sup>82</sup>



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# Endnotes

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