BIOPHARMA THOUGHT LEADERS

PARTNERING: A KEY PILLAR BOLSTERING PFIZER'S INNOVATION PIPELINE

Pfizer's Centers for Therapeutic Innovation and other partnership initiatives champion emerging science and help stakeholders realize the potential of their innovations.

Successful drug development requires a rich and collaborative ecosystem involving many counterparts. It requires sharing information on everything from target selection to manufacturing platform technology, which drives researchers in biopharma, academia, start-ups, and biotech companies to collaborate. This joint vision keeps the landscape competitive as innovators seek precious opportunities to differentiate their offerings to prospective partners.

Pfizer, a leading company in the partnering space, offers a customized, two-pronged approach. One team focuses on partnering defined clinical assets. Another seeks cutting-edge, pre-clinical science and breakthrough technologies from academia, public-private partnerships and biotech. For the latter, identifying the most promising nascent external opportunities, matching them with the optimal collaboration vehicle to meet the diverse interests of potential partners, and translating these into robust drug development programs are among the key challenges.

Uwe Schoenbeck, Pfizer's Chief Scientific Officer for Emerging Science and Innovation (ES&I), said "Partnering is key for us, around 40% of our portfolio has been either externally sourced or critically enabled through partnerships. Securing clinical stage assets is critical for the short to midterm, but it is arguably just as important for an innovative biopharma to access cutting-edge science, breakthrough technologies and early-stage top quality assets to support our long-term success and leadership. We seek collaborations with academic medical centers and their investigators, and biotech companies in which both parties bring complementary capabilities to achieve something neither of us could do alone."

"To achieve this goal, Pfizer implemented a number of unique partnering vehicles, such as the Centers for Therapeutic Innovation (CTI)," added Schoenbeck, connecting internal resources and capabilities with academic discoveries to deliver a novel, transformative pipeline. CTI helps academic investigators navigate early translational challenges, providing hands-on support from industry experts. "CTI works with academic PIs [principal investigators] to translate their innovative ideas for therapies into robust drug development programs," he explained. "It combines the external investigator's insights into novel science with complementary internal drug development expertise and capabilities to advance breakthrough therapies much faster and more robustly than could be done otherwise. Joint project teams create focused research plans based on agreedupon scientific goals and deliverables, leveraging



the complementary expertise of the PI and Pfizer researchers throughout the length of the project. Engaging PIs in the industrial drug development process with an experienced company like Pfizer is a significant attraction to many investigators."

Each CTI project is led by a Pfizer scientific champion who uses internal research capabilities to address early translational challenges. CTI scientists engage from project conception to exit, ensuring transparent decision-making and appropriate engagement of Pfizer enterprise resources, expertise and capabilities. "We have a portfolio of around 30 CTI projects at any given time," said Schoenbeck. "Pfizer has now advanced six CTI programs into the clinic, soon to be seven, of which three are in phase 2 clinical trials."

The CTI model is based out of four labs in New York, Boston, San Francisco and San Diego. Joe Dal Porto, Vice President and Head of CTI California, said "Through an open door policy, these labs

Emerging Science & Innovation: an external-facing partnering engine

Pfizer's Emerging Science and Innovation (ES&I) team is the dedicated partnering organization within Pfizer's Worldwide R&D and Medical (WRDM) organization, tasked with sourcing and advancing cutting-edge science in collaboration with academics and biotech. ES&I seeks:

- External innovative technologies and early-stage opportunities that may yield near-term pipeline impact. These are sourced by *Emerging Science Leads (ESLs)* working in partnership with Business Development and Legal colleagues.
- Novel drug targets that may translate to breakthrough therapies. *Pfizer's Centers for Therapeutic Innovation (CTI)* work together with principal investigators at leading global academic medical centers to translate early scientific concepts into robust drug development programs. The CTI pipeline is further enhanced through genetics-based insights from Pfizer's *Target Sciences (TS)* team, which delivers novel target hypotheses through human and murine genetics.
- Emerging innovations of the future. Seed and venture investments through the *Pfizer Ventures* team nurture external science in nascent biotechs that pursue differentiated and transformative science with future potential to enhance Pfizer's core areas of therapeutic interest. Pfizer also actively participates in public-private partnerships and consortia to explore even earlier stage emerging science and technologies in partnership with academia, biotech and peer pharma companies to help shape the scientific and health-care ecosystem.

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Uwe Schoenbeck, Senior Vice President and Chief Scientific Officer

> Emerging Science & Innovation (ES&I)

> > Pfizer

promote investigators to work alongside our scientists. Likewise, our scientists can work with the external institution or PI anywhere in the world."

"PIs value the level of involvement," David Shields, Vice President and Head of CTI New York and Boston, added "One of CTI's most unique benefits is the flexibility to tailor each collaboration to best serve mutual goals and access a range of partnering vehicles to support program progression. We can offer multiple options to the investigators; they can decide whether they would prefer early-stage research funding, longer term co-drug discovery and development collaborations to translate novel therapeutic concepts, or, potentially, support to develop a NewCo."

In addition to recurrent Calls-for-Proposals and targeted networking within partner institutions, CTI's portfolio is also driven by ES&I's Target Sciences (TS) team. Applying genetics-driven drug target identification and validation as well as dedicated biomarker and diagnostics capabilities, TS enables the maturation of the most promising early pipeline targets.

Locating the best science

Identifying the right partnering opportunity—particularly for less known early science developments and technologies—remains a key challenge before a drug development program can even start. ES&I approaches this by:

- Emerging Science Leads (ESLs): A team of seasoned PhD/MDs search for and evaluate opportunities from academia and biotech for Pfizer's R&D organization. ESLs are highly experienced in the relevant disease area and embedded within the respective therapeutic areas, resulting in high strategic alignment of the opportunity being sourced and avoiding opportunities that are not a strategic fit. "It's easier to identify what's complementary if you have an understanding of the therapeutic area and what's not on the typical partnering list that might be cutting edge," said Schoenbeck.
- Pfizer Ventures: The ventures team nurtures cutting-edge science through seed and equity investments. "Working with business development colleagues under the Pfizer Ventures umbrella, we have expanded our equity investment vehicles from a classical corporate venture format, to include seed investments in new corporations, and the formation of new companies, such as the recently launched FoRx Therapeutics-focused on DNA Replication Stress, a subcategory of DNA Damage Response, an emerging science area within CTI and of strategic interest for our oncology and rare disease research units," explained Denis Patrick, Vice President and Head of Partnering Innovation. "We are doing this in areas that are core to Pfizer's R&D interests today but also in up-and-coming areas, which allow us to nurture science that might not be ready for partnering today but has high potential." Patrick's team also leads Public Private Partnership efforts with the same early engagement goal. For example, Pfizer is an active player in the European Innovative Medicines Initiative (IMI). "We have about 45 IMI partnerships across Pfizer, with projects in areas as diverse as digital pathology, green chemistry, patient reported outcomes, pediatric oncology and, most recently, COVID-19," he said.



Remaining competitive

"Pfizer's early-stage partnering model allows it to stay competitive in a rapidly evolving ecosystem, and to place strategic bets in emerging areas," said Schoenbeck. "Our embedded teams can see outside opportunities that might not be an immediate fit but could advance the field. It is the seamless integration of the various partnering vehicles that uniquely positions Pfizer in the competitive landscape of early stage partnering, where you can see seed and equity investments translating into licensing deals or acquisitions and early-stage partnerships with academia yielding cutting-edge, differentiated medicines."

One example is Pfizer's early entry to the gene therapy market. Before any significant approvals had been realized, the company recognized that the technology platform and therapeutic modality held significant potential and should be applied to rare diseases first, which led to its 'Build, Buy, and Partner' collaboration and acquisition strategy.

Pfizer's partnership with Spark Therapeutics developed a potential therapeutic for hemophilia B—an area in which it already had a strong presence. It then acquired Bamboo Therapeutics with its AAV9-based gene therapy candidate for neuromuscular diseases (such as Duchenne muscular dystrophy and Freidreich Ataxia), and a clinical-scale adeno-associated virus vector manufacturing process. Pfizer recently partnered with Sangamo Therapeutics on a hemophila A gene therapy candidate, currently in phase 3 clinical testing, and also with Vivet Therapeutics for Wilson Disease, a rare inborn error of metabolism. Pfizer complemented these externally-sourced efforts with significant internal investments, from home grown gene therapy projects to manufacturing capabilities.

Seng Cheng, Pfizer's Chief Scientific Officer for Rare Disease, said, "In the last few years we successfully advanced three gene therapy programs into clinical trials and are working in partnership to initiate another in the coming year. We wish to build upon this pipeline of initiatives, and that is where the various ES&I channels play a big role in continuing to source opportunities."

Barbara Sosnowski, Vice President and Head of the ESLs, added, "Our ESLs come with an entrepreneurial spirit, building excitement and resilience. We interface with academic medical centers and biotech companies looking for cutting-edge science, network with investigators and uncover every stone looking for new opportunities. We respect that the partnering entity strongly believes in its own research and understand how it feels to bring something up from the ground and into a larger company."

The Rare Disease team also wants to invest in novel adeno-associated virus vectors that offer improved tissue tropism or potency, enable gene therapy as an option for other diseases, and address existing technology gaps such as host immunity issues associated with viral vectors. "We'd like to identify additional adjacent diseases that are aligned with our current therapeutic areas of focus to bring in-house, or develop internally, leveraging the gene therapy knowledge and expertise that we have developed through our current suite of gene therapy tools," said Cheng. Pfizer also plans to build upon its manufacturing strengths to offer more efficient, cost effective and higher yielding processes for an increased number of gene therapies.

"Pfizer stands out because of our early commitment to gene therapy as one key part of the armamentarium of potential treatments for rare diseases," Cheng concluded. "We are very proud that we can embark on additional programs now and support their development across the preclinical, early clinical and commercial axis."

Pfizer's multi-layered partnering model, driven by ES&I, enables it to lock into a multitude of innovator discoveries from academia to biotech, recognizing talent and promise early on, and providing stakeholders with a committed partnership throughout. Meanwhile, networking and relationship building yields fresh opportunities, said Schoenbeck, citing its relationship with German biotech BioNTech as an example. "When we first connected years back we discussed partnerships in oncology, but a couple of years later, we found common ground in a partnership around a flu vaccine candidate—and together we are now one of the leaders in developing a potential vaccine against COVID-19, illustrating that you never know where relationships can lead you."

