



This strategy is central to Vertex and has fundamentally shaped the company's pipeline of R&D programs. It starts with a scientific problem waiting to be solved. "We first 'crack the biology' of a disease, meaning we ensure our biological target plays a causal role in the disease. Then we 'pour on the chemistry or technology' to find potential treatments," says Kewalramani. "We spend a lot of time and effort validating our laboratory assays and clinical biomarkers upfront, which gives us higher confidence that a potential therapy will succeed when we investigate it in the clinic."

Investing in scientific research is clearly where the company is focused. In 2019, 73% of the company's operating expenses were dedicated to R&D, a disproportionately large amount when compared to the average biotech and pharma companies in the industry. In addition, three out of five employees work in R&D, which Altshuler credits as the most important aspect of drug discovery.

"Discovering potential new medicines is the ultimate team sport," says Altshuler. "It takes hundreds of scientists and researchers working together to bring a new medicine to patients who are waiting."

Team Impossible is on the case

Indeed, "I often refer to our teams as 'Team Impossible,' because no matter how big the challenge, they always find solutions that seem to defy what's possible," says Kewalramani. "This sense of urgency is incredibly important to the people living with serious diseases."

Vertex spearheads novel systems and processes to foster this velocity and tenacity. For example, in 2015, Vertex became one of the first companies to implement drug product continuous manufacturing (CM) technology in the development and production of its investigational and commercial medicines. With CM, raw materials are added into one continuously running system, and quality control takes place throughout the production process. "Continuous manufacturing results in faster, more streamlined manufacturing process development and scale-up," says Stuart Arbuckle, Vertex's chief commercial officer. "We've successfully integrated this technology into our process development, clinical and commercial supply production processes."

Another way Vertex reduces development timelines is by doing clinical studies and other development activities in parallel, where possible, instead of one at a time. "We're always looking for ways we can do multiple things at the same time, because having these faster timelines gives us the potential to get

medicines to patients quicker, and the sooner we can get medicines to patients the better," explains Carmen Bozic, Vertex's chief medical officer.

Vertex's innovations aren't just happening in the lab or in clinical studies. Nia Tatsis, Vertex's chief regulatory officer, views the company's partnerships with regulators as a critical piece of the puzzle. "Part of my goal is to ensure regulations are keeping pace with scientific innovation," she says. "Our science is evolving rapidly, and we need strong working relationships and trust with regulators to achieve our shared goal of bringing new medicines to patients who need them."

None of these advancements would be possible if not for the enterprise's prime directive: "We have developed a very special, inclusive culture at Vertex," Kewalramani shares. "More than half of our global employees are women and five out of 10 of our directors are diverse on a gender or ethnic basis—and it's that diversity of thought and experience that ensures we make the best decisions for patients and our business."

To infinity and beyond: Tackling the next set of disease targets

"Our mission is to use scientific innovation to bring treatment options to people with serious diseases. So we're applying what we've learned in CF to a number of other serious diseases in which we understand the causal human biology and have validated targets and biomarkers with high fidelity from bench to bedside, such as sickle cell disease, alpha-1 antitrypsin deficiency, and Duchenne muscular dystrophy," says Kewalramani. "We're leveraging multiple therapeutic modalities such as cell therapies, gene editing, and mRNA [messenger RNA] therapies."

Vertex continuously asks the following questions: What else can we do to advance medicines? Where else can we apply our research in helping patients? How can we bring medicines to patients faster? By using the power of biology, chemistry, emerging technologies, creativity, and teamwork to address serious diseases, Vertex will continue to pioneer advances in science and medicine in the service of patients who are waiting for them.

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