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Our View on Drug Pricing

At Vertex, our mission is ambitious: to discover medicines that can prevent, cure or fundamentally change the outcomes of serious diseases for patients and their families. We are relentless. We go all in. For more than 20 years we have worked on cystic fibrosis (CF), and today we have four approved breakthrough medicines with the potential to eventually treat the underlying cause of CF in up to 90% of all patients around the world. We are on track with other investigational therapies to potentially transform the treatment of multiple other serious diseases like sickle cell disease, diabetes and muscular dystrophy.

But discovering transformative medicines is risky and expensive. Industry wide, more than 90% of all medicines that start clinical trials fail to reach approval. Our research and development programs cost more than \$1 billion a year. And we too fail many times before we succeed.

Our philosophy on pricing reflects a few basic principles:

- 1. We believe that only by investing heavily in scientific innovation will we transform the treatment of serious diseases and radically improve the lives of patients and their families. We invest the vast majority of our operating expenses (more than 70%) in R&D to discover new medicines. Nearly two-thirds of employees at Vertex are directly involved in drug discovery and development.
- 2. The prices of our medicines reflect their benefits to patients. They have transformative benefits for people with serious diseases and are often the first of their kind. Prices also reflect the size of the patient population being treated. The cost of drug discovery is high whether you are treating 10 patients or 1 million patients.
- 3. It is our revenues from existing products that fund our ongoing research in current and future disease areas. Investigative treatments like innovative cell therapies that could potentially cure type 1 diabetes. Gene editing programs that could potentially cure sickle cell disease and beta

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7. Drug costs get a lot of attention in comparison to other parts of health care systems. But medicines account for less than 15% of total U.S. health care spending, and this is also consistent across many OECD countries. On the other hand, to date transformative medicines have proven to be a highly effective way to reduce the overall costs that threaten to bankrupt health care systems, while at the same time improving patient outcomes.

Health care systems and governments must continue to support and reward both basic scientific research and innovation in drug discovery in order to nurture the biomedical ecosystem and ensure the discovery of the next set of breakthrough cures for diseases like diabetes, Alzheimer disease and cancer, and thereby improve and potentially extend patients' lives and reduce health care costs.

For our part, Vertex will continue to be relentless in tackling serious diseases, bringing innovative new treatments to patients and ensuring patients have access to these medicines.