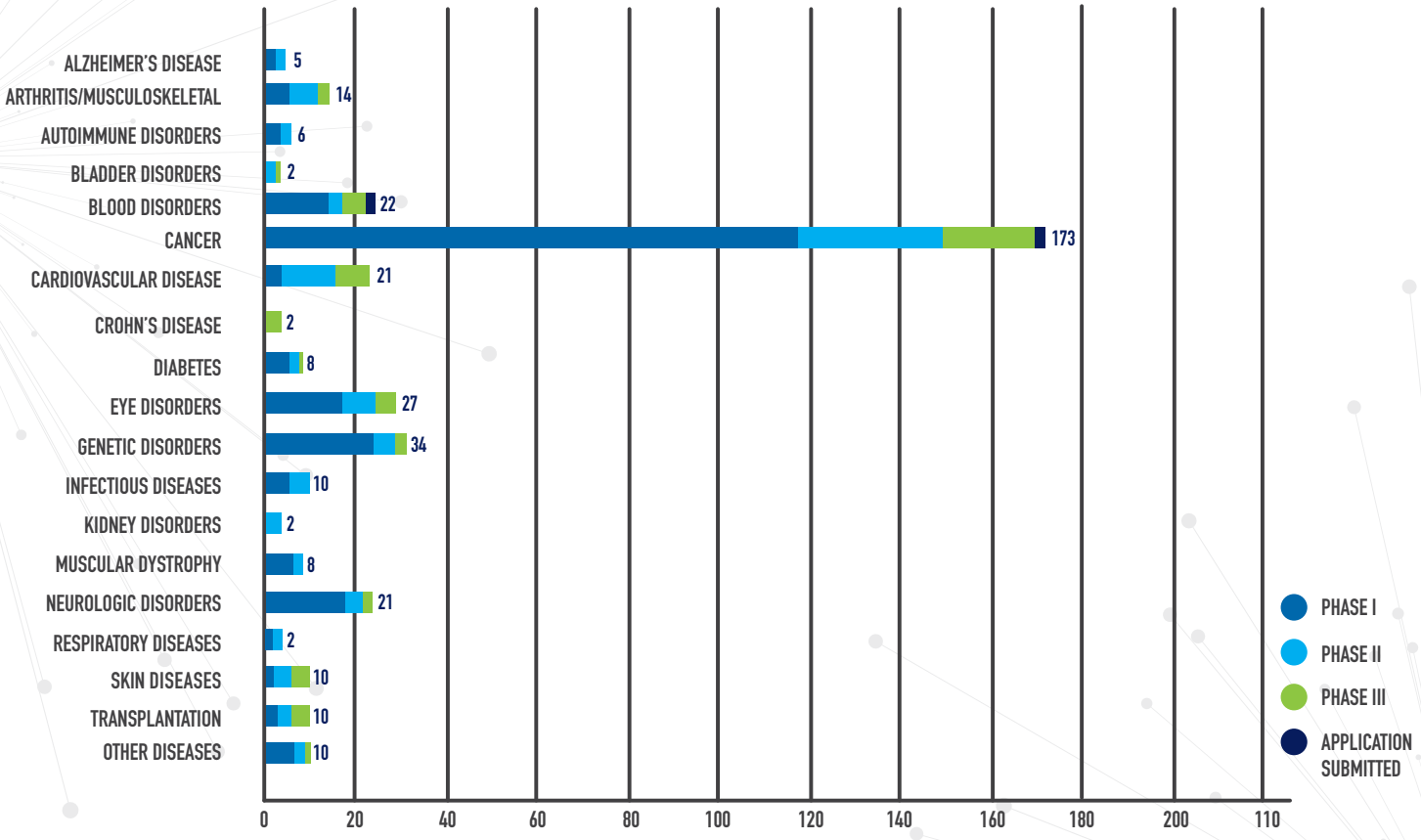
HOW THE U.S. HEALTH CARE SYSTEM CAN BETTER ACCOMMODATE CELL AND GENE THERAPIES

Cell and gene therapies use an individual's cells and genes to treat disease. While this sounds simple, the reality could not be more complex from a scientific and clinical standpoint. Cell therapy involves taking cells from the patient or a donor and genetically altering and reinserting into the patient to treat the underlying cause of the disease. Gene therapy treatment involves making an addition to, silencing or altering a gene. These treatments are often referred to together as they both involve manipulating a person's genes at the molecular level.

CELL AND GENE THERAPIES OFFER TREMENDOUS PROMISE FOR PATIENTS

Scientists first began researching the potential of gene therapy in the 1980s. Today, there are nine cell or gene therapy products approved in the United States and scientists and researchers at America's biopharmaceutical companies are developing nearly 400 cell and gene therapies to treat cancers, neurological diseases and more. These therapies offer the potential to cure previously incurable diseases and to fundamentally alter the trajectory of many other life-threatening conditions, including many rare diseases. In fact, a third of cell and gene therapies in development are for rare diseases.[1]

Medicines in Development by Disease and Phase



* SOME MEDICINES ARE IN MORE THAN ONE CATEGORY.

For example, beta thalassemia is a disease where these therapies could have a transformative impact not only on the lives of patients but on the health care system. Impacting just 2,000 Americans, beta thalassemia occurs when hemoglobin – the protein that carries oxygen around the body – doesn't function properly, resulting in malformed blood cells and anemia. Patients require lifelong treatment starting from birth, often involving chronic blood transfusions and burdensome iron chelation therapy. Not surprisingly, the disease is associated with significant patient burden and costs. Potential gene therapies currently in the late stages of clinical development can dramatically reduce this burden by about 41% per patient over five years, leading to $196,000 in reduced blood transfusion therapy costs, $111,000 in reduced iron chelation therapy costs and $18,000 in reduced costs resulting from missed work for caregivers and patients with the disease.

These therapies are the result of decades of research and are incredibly challenging to research, develop, manufacturer and deliver to patients. Yet, they offer the potential to have a dramatic and immediate impact on patients' lives and our health care system.[2]

CHALLENGES AND OPPORTUNITIES

These therapies harness the latest scientific advances, but our current health care system is not structured to handle potential one-time therapies. While patients with commercial insurance have been able to access certain new treatments, the current Medicare payment system is not well suited to pay adequately for innovative cell and gene therapies, often preventing beneficiaries from accessing these breakthroughs. Though patients with commercial insurance have been able to access these treatments, current payment challenges prevent innovative contracts that would help patients more efficiently access these treatments and enable our reimbursement system to evolve with the pace of scientific advancement.

PhRMA member companies are focused on identifying ways to further evolve the health care system to ensure continued capacity for these innovative therapies.

1. Biopharmaceutical companies are continuing to explore existing and new technologies to more efficiently and cost effectively develop and manufacture cell and gene therapies, including exploring the feasibility of manufacturing therapies at the point of care.

2. Americans need a health system that values treatments based on patient outcomes. Biopharmaceutical companies are increasingly exploring innovative contracts—also known as value-based contracts or alternative financing arrangements—in which payment for new treatments is tied more closely to patient outcomes. Research shows that contracts may reduce health system and patient out-of-pocket costs and improve patient access and health outcomes.[3]

3. Our health care system needs to continue to explore new approaches to financing these medicines. Just as there won't be one size fits all gene and cell therapies, there is not a one size fits all approach to payment. Biopharmaceutical companies and payers are exploring a range of creative innovative contracting approaches that can include outcomes-based arrangements, extended payment plans and annual subscription plans.[4]

As stated by the FDA, some of these therapies "are almost certainly going to change the contours of medical practice, and the destiny of patients with some debilitating diseases."[5] It is critical that we work together to develop a coverage and payment system that ensures timely patient access, manages short-term affordability challenges and continues to foster the development of these new treatments.

[1] PhRMA. Medicines in Development: Cell and Gene Therapies. March 2020.

[2] Health Advances. "Economic Burden of Hematological Diseases and Cost-Offset Potential of Novel Gene Therapies," August 2020.

[3] National Pharmaceutical Council. Value-Based Contracts. Available from: https://www.npcnow.org/issues/access/provider-reimbursement/risk-sharing-agreements

[4] PhRMA. Value-Based Contracts: 2009 - Q4 2019. February 2020. Available from: https://www.phrma.org/Fact-Sheet/Value-Based-Contracts-2009-Q4-2019

[5] FDA Statement, "Statement from FDA Commissioner Scott Gottlieb, M.D. on agency's efforts to advance development of gene therapies," July 11, 2018.