AMERICAN SOCIETY of GENE & CELL THERAPY

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Outside Witness Testimony for Fiscal Year 2020 Appropriations
Submitted by the American Society of Gene & Cell Therapy
Prepared for the Subcommittee on Labor, Health and Human Services, Education, and Related Agencies regarding appropriations for the US Department of Health and Human Services, National Institutes of Health

June 3, 2019

The Honorable Roy Blunt
Chairman, Labor, Health and Human Services, Education, and Related Agencies
United States Senate
260 Russell Senate Office Building
Washington, DC 20510

The Honorable Patty Murray
Ranking Member, Labor, Health and Human Services, Education, and Related Agencies
United States Senate
154 Russell Senate Office Building
Washington, DC 20510

Dear Chairman Blunt, Ranking Member Murray, and Subcommittee Members:

Thank you for the opportunity to provide this testimony on behalf of the American Society of Gene & Cell Therapy (ASGCT). ASGCT is a membership organization consisting of scientists, physicians, and other professionals involved in the gene and cell therapy fields in settings such as universities, hospitals, government agencies, foundations, and biotechnology and pharmaceutical companies.

The Society respectfully requests robust FY2020 appropriations to the National Institutes of Health to support the biomedical research that can advance future gene and cell therapies. Further funding of gene and cell therapy research has the potential to accelerate the discovery and clinical application of more safe, effective, and innovative genetic and cellular therapies to alleviate and ease human disease, which is a core component of the mission of ASGCT.

Significance of NIH Research Funding for Gene and Cell Therapy

NIH funding is crucial to support basic research on biological targets as well as applied research on new molecular entities, which both contribute to new therapeutic approvals.[1] NIH funding contributed to published research associated with every one of the 210 new drugs approved by the Food and Drug Administration from 2010 – 2016.[1] The development of new therapeutics therefore relies upon this investment, in order to accelerate the development of new gene therapies for treatment of as many genetic diseases as possible.

[1] Cleary, E.G., Beierlein, J.M., Khanuja, N.S., McNamee, L.M., Ledley, F.D. (2018). Contribution of NIH funding to new drug approvals. In Snyder, S. H. (Ed.) *Proceedings of the National Academy of Sciences, 201715368,* doi: 10.1073/pnas.1715368115.

For example, grants provided by the federal government have funded research that has led to clinical trials to treat sickle cell disease. Sickle cell disease is caused by a single gene mutation that results in a lack of production of beta globin, a component of hemoglobin (a protein in red blood cells). This lack of beta globin causes rigid, sickle-shaped red blood cells that do not carry oxygen as well as normal cells.[2] Reduced blood flow and oxygen can cause weakness, fatigue, slowed growth, severe pain and other serious complications, such as harm to a patient's liver, brain, eyes, and lungs, among other organs.[3]

Cincinnati Children's Hospital, which receives the majority of its funding through the NIH and ranks second among pediatric hospitals in funding from the agency, has established a Comprehensive Sickle Cell Center to combat this disease.[3] ARU-1801, originally developed in Dr. Punam Malik's laboratory at Cincinnati Children's Hospital, is an investigational gene therapy for sickle cell disease that aims to increase functioning red blood cells by removing a patient's own stem cells, inserting a functioning gene into the cells outside the body, and delivering the cells back into the patient.[4] Preliminary findings in Phase 1/2 clinical trials showed a substantial reduction in disease symptoms—near elimination of chronic pain and sickling events, and improved anemia.[5]

Federally-funded research has benefits that stretch beyond the scope of clinical development. In addition to its direct contributions to gene therapy-related research, NIH-funded research is responsible for the creation of approximately 440,000 jobs and $69 billion in economic stimulation.[6] Furthermore, studies show that NIH investments in biomedical research stimulate increased private investment; with every dollar of increase in public clinical research stimulating $2.35 of industry investment at 3 years.[7] This economic stimulation is even higher for gene-related research, with a federal investment of $3.8 billion in the Human Genome Project from 1988 to 2003 helping to drive $796 billion in economic output, which is a return of $141 for every $1 invested.[8]

Need for Additional Gene and Cell Therapy Research

Progress in clinical trials of gene therapies and gene-modified cell therapies exemplify the vast medical progress that NIH research has contributed to in these areas, which provides hope for the future. However, considerable additional scientific study will be necessary for gene and cell therapies to reach their potential to transform the lives of patients with multiple additional diseases. Many of the diseases

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[2] Sickle Cell Disease. (n.d.). Retrieved from https://www.nhlbi.nih.gov/health-topics/sickle-cell-disease.
[3] Funding Growth Fuels Discovery. (n.d.). Retrieved from https://www.cincinnatichildrens.org/research/cincinnati/ccrf/funding.
[4] Biopharmaceutical Drug Development. (2019). Retrieved from https://roivant.com/#pipeline.
[5] Early Clinical Trial Data Show Gene Therapy Reversing Sickle Cell Anemia. (2018). Retrieved from https://www.cincinnatichildrens.org/news/release/2018/gene-therapy.
[6] United for Medical Research: NIH's role in sustaining the U.S. economy: 2018 update. http://www.unitedformedicalresearch.com/wp-content/ uploads/2018/ 02/NIHs-Role-in-Sustaining-the-U.S.-Economy-2018- Update-FINAL.pdf.
[7] Heymach, J., Krilov, L., Alberg, A., Baxer, N.,Chang, S. M., Corcoran, R., … Burstein, H. Clinical Cancer Advances 2018: Annual Report on Progress Against Cancer From the American Society of Clinical Oncology. *Journal of Clinical Oncology 2018 36*(10), 1020-1044.
[8] Accelerating Biomedical Research Act, H.R. 5455, 115th Cong. (2018).

for which gene therapy offers great promise are rare inherited disorders. Of the 7,000 rare diseases that exist, 95 percent have no current treatment.[9]

Continued strong funding for multiple institutes and centers of the NIH can support gene and cell therapy research to address this immense unmet need and the resulting human and economic costs of diseases such as hemophilia, muscular dystrophy, and retinal disorders that collectively impact the lives of 10 percent of the US population.[10] Children with some hereditary diseases cannot walk, or even breathe or swallow on their own. Tragically, many of these children die young or become severely disabled by adolescence. For diseases with longer life expectancy, such as hemophilia, patients face a lifetime of intensive and expensive medical care. For example, the average lifetime cost of treating hemophilia A with bypassing agent prophylaxis can range from $90 million to $99 million.[11] Developing potentially durable, often one-time gene therapies for these diseases will require significant research funding, which will ease or potentially end the human suffering, and in some cases the high current medical costs, that they currently incur.

Appropriations Request

1. Since gene and cell therapies are types of regenerative medicine, ASGCT is grateful for the funding authorized by the 21st Century Cures Act for the Regenerative Medicine Innovation Project (RMIP).[12] Appropriations of a total of $22 million in FY2017 – FY2019 for RMIP, as authorized, are greatly appreciated, resulting in funding of 16 grants over the last two years.[13] The Society requests that the $8 million authorized by the Cures Act for FY2020 is appropriated for this initiative.
2. While NIH funding increases have been generous over the past four years, the need remains to maintain global leadership in medical innovation and to compensate for funding that still lags behind funding levels at the end of the budget-doubling initiative which ended in 2003, when adjusted for biomedical research inflation.[14] This era resulted in the grant application success rate diminishing to below historic averages. Without the current budget restraints, the grant application success rate could be closer to one in every three applications. However, fiscal year 2018 produced a grant application success rate of 22.7 percent.[15] Increases in funding to the NIH in general, and to the gene and cell therapy fields in particular, need to continue to support the potential progress in the development of these transformative treatments. The Society also requests at least a $2 billion increase in NIH funding for FY2020, as has also been proposed by the House Labor, Health and Human Services, Education and Related Agencies Appropriations Subcommittee.

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[9] Institute of Medicine (US) Committee on Accelerating Rare Diseases Research and Orphan Product Development; Field, M.J., & Boat, T.F., editors. Rare Diseases and Orphan Products: Accelerating Research and Development. Washington (DC): National Academies Press (US); 2010. Available from www.ncbi.nlm.nih.gov/books/NBK56189. doi: 10.17226/12953.

[10] Maude, S., Laetsch, T., Buechner, J., Rives, S., Boyer, M., Bittencourt, H., … Baruchel, A. (2018). Tisagenlecleucel in children and young adults with B-cell lymphoblastic leukemia. *N Engl J Med 378,* 439-448.

[11] Pearson, S. (2018). *Can We Determine "Value-based" Prices for Gene Therapies?* Lecture presented at ASGCT Value Summit, Washington, DC.

[12] 21st Century Cures Act, H.R. 34, 114th Cong. (2015).

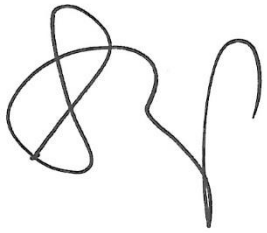
[13] Funding Opportunities. (2018). Retrieved from https://www.nih.gov/rmi/funding-opportunities.

[14] Budget. (2019). Retrieved from https://www.nih.gov/about-nih/what-we-do/budget.

[15] Success Rates - NIH Research Portfolio Online Reporting Tools (RePORT). (2019). Retrieved from https://report.nih.gov/success_rates/.

In conclusion, because NIH funding can contribute to the development of new gene and cell therapies to treat diseases with great unmet medical need, ASGCT encourages the Senate Committee on Appropriations, Subcommittee on Labor, Health and Human Services, Education, and Related Agencies to provide robust appropriations in its FY2020 funding to the many institutes and centers of the NIH that engage in gene and cell therapy related research. The Society also advocates for continued separate, specific appropriations to continue to fund the Regenerative Medicine Innovation Project. We appreciate your consideration of these comments.

Sincerely,



Guangping Gao, PhD
President