New Analysis by Tufts' NEWDIGS Shows that Durable Cell and Gene Therapies Have Substantially Higher Clinical Success Rates than Other Treatments

Carlsbad, CA – October 11, 2023

Findings to be shared during ARM's annual Cell & Gene Meeting on the Mesa
Read the Research Brief Here

The Alliance for Regenerative Medicine (ARM), the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines, today announced new analysis showing that durable cell and gene therapies have clinical success rates between two and three times higher than other types of treatments. NEWDIGS of Tufts Medical Center conducted the research – supported by ARM — by using its proprietary data set of durable cell and gene therapy clinical trials. NEWDIGS will present the research today at ARM's annual Cell & Gene Meeting on the Mesa in Carlsbad, CA.

"Cell and gene therapies are transforming outcomes for people living with serious and sometimes fatal conditions," said Mark Trusheim, Strategic Director, NEWDIGS. *"This analysis provides evidence that once cell and gene therapies enter clinical development they are not only more likely to ultimately gain FDA approval than other candidate medicines but are also more likely to advance at nearly every phase of the clinical development process."*

Hematologic Cancers
CAR-T/TCR therapies for hematologic cancers entering clinical development are three times as likely to gain approval by the U.S. Food & Drug Administration (FDA) than the average oncology drug, based upon comparing success rates from all CAR-T/TCR clinical trials from 1988 through 2020 reported in clinicaltrials.gov to the success rates calculated in a previous report by BIO, "New Clinical Development Success Rates 2011-2020 Report." Further, as compared to previous estimates of average oncology treatments, CAR-T/TCR therapies outperform the average oncology drug from Phase 2 through FDA application review. The largest success rate difference for CAR-T/TCRs occurred in Phase 3 (100% progression to FDA application review for CAR-T/TCR vs. 48% for oncology drugs). The comparative analysis shows that the average CAR-T/TCR therapy has a 17% chance of receiving FDA approval once it enters Phase 1 versus a 5.3% chance across all oncology.

Rare Diseases
Once entering Phase 1, rare disease durable gene therapies are two to three-and-a-half times more likely to gain FDA approval compared to the average drug across all modalities, again per comparisons of all durable cell gene therapies in clinical trials from 1988 through 2020 to the BIO report and IQVIA's report "Global Trends in R&D 2023." In comparing success rates by phase of clinical development, gene therapies outperform average drugs at every step. The analysis shows that the

average rare disease gene therapy has a 28% chance of receiving FDA approval once it enters Phase 1.

Compared to average drugs included in the BIO Report, orphan gene therapies had:
• 48% higher success rate in Phase 1 clinical trials
• 65% higher success rate in Phase 2 clinical trials
• 30% higher success rate in Phase 3 clinical trials
• 10% higher success rate in completing a New Drug Application (NDA) or Biologics License Application (BLA)

Similar results were found in comparing gene therapy success rates with average drugs via the IQVIA report:
• 37% higher success rate in Phase 1 clinical trials
• 23% higher success rate in Phase 2 clinical trials
• 12% higher success rate in Phase 3 clinical trials
• 12% higher success rate in completing a New Drug Application (NDA) or Biologics License Application (BLA)

In assessing clinical success rates within therapeutic areas commonly addressed by gene therapies (hematology, autoimmune, metabolic, neurology, ophthalmology), gene therapies are more than twice as likely to gain FDA approval when they enter Phase 1 compared to the average for treatments in those areas as reported by BIO, again outperforming in every phase of clinical development.

"Cell and gene therapies are truly revolutionizing medicine by durably treating, and potentially curing, the root causes of disease. This new analysis by NEWDIGS shows that the first generation of these innovative treatments is substantially outperforming other, less targeted treatments in the clinic – and this is just the beginning. As the clinical pipeline of genetic medicines and engineered cell therapies continues to grow, we look forward to even greater impact for patients," said ARM's Chief Executive Officer Tim Hunt.

See the full Durable Cell and Gene Therapy Comparative Success Rates Analysis research brief here. You can also view a fireside chat with Mark Trusheim via online streaming by registering for a complimentary media pass for Meeting on the Mesa.

About the Durable Cell and Gene Therapy Comparative Success Rates Analysis
Data were sourced through NEWDIGS' FoCUS Project's Pipeline Analysis Model, which accounts for all durable cell and gene therapy clinical trials from 1988 through 2020 reported to clinicaltrials.gov. To gain a better understanding of the success rates of cell and gene therapy clinical programs, NEWDIGS analyzed success of cell and gene therapies at different phases of clinical development – from phase 1 through application submission to the U.S. FDA – in hematologic cancers and rare conditions. Those success rates were then compared to recent published findings on drug clinical development success rates: "New Clinical Development Success Rates 2011-2020 Report" by BIO and IQVIA's "Global Trends in R&D 2023."

Every medical condition, and therapy designed to treat it, is unique. Data included in these analyses are averaged across many therapies and medical conditions; analyses for specific treatments should be completed individually.

About NEWDIGS at Tufts Medical Center
NEWDIGS is dedicated to improving health outcomes by accelerating appropriate and timely access for patient to biomedical products – in ways that work for all stakeholders. NEWDIGS designs, evaluates, and catalyzes the real-world implementation of system innovations that are too complex and cross-cutting to be addressed by a single organization or market sector. Its members include global leaders from patient advocacy, payer organizations, biopharmaceutical companies, regulatory agencies, clinical care, academic research, and investment firms. For more information, visit newdigs.tuftsmedicalcenter.org.

About the Alliance for Regenerative Medicine
The Alliance for Regenerative Medicine (ARM) is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. As a community, ARM builds the future of medicine by convening the sector, facilitating influential exchanges on policies and practices, and advancing the narrative with data and analysis. We actively engage key stakeholders to enable the development of advanced therapies and to modernize healthcare systems so that patients benefit from durable, potentially curative treatments. As the global voice of the sector, we represent more than 400 members across 25 countries, including emerging and established biotechnology companies, academic and medical research institutions, and patient organizations.