
**ISCT revises cell and gene therapy industry forecast
following FDA Novartis CTL019 approval**

International society forecasts significant investment and funding throughout entire cell and gene therapy sector

Vancouver, Canada, August 30, 2017 - The International Society for Cellular Therapy (ISCT), the global professional society of clinicians, researchers, regulatory specialists, technologists and industry partners in the cell therapy sector, today announces its revised forecast for the CAR-T and cell therapy industries. The forecast follows the US approval by the FDA of the Novartis CAR-T cell therapy CTL019. It encompasses the expected cross-industry reaction to the approval, and envisions the next stages in the development of cell therapies and the industry.

The Novartis cell therapy FDA approval will result in the first gene modified cell therapy in the US, and the first CAR-T therapy approved internationally. ISCT believes this is a considerable milestone reached by the cell therapy sector and direct evidence of the commercializing potential of CAR-T therapies. The approval demonstrates to all investors and public sector and government grant and funding providers that cell therapy is now a sector that has emerged today, not evolving in the future.

ISCT believes that this approval will undoubtedly have a considerable reaction with significant ripple effects right across all stakeholders involved in the cell therapy field. The organization forecasts significant reaction from investors and considerably increasing investment right through the therapeutic development pipeline, from academic research to early stage pre-clinical and clinical development right through to late stage pre-commercialization trials. ISCT also foresees a considerable increase in investment for support industries, from CROs and CMOs to equipment, reagent, and material suppliers, logistics and tracking in the cell and gene therapy space.

“There are now upwards of 40 companies developing redirected T cells or NK cells for therapeutic use. There are also over 800 cell therapy clinical trials currently underway, and a considerable pool of research and pre-clinical work right across the cell therapy sector,” said Bruce Levine, Ph.D., ISCT Commercialization and Immuno & Gene Therapy Committees, Barbara and Edward Netter Professor in Cancer Gene Therapy, University of Pennsylvania Perelman School of Medicine. Dr. Levine and colleagues at the University of Pennsylvania and the Children’s Hospital of Philadelphia led research, development, and clinical trials of the new therapy that was licensed to Novartis. “International investment and grants are as critical for early research as it is for late-stage clinical development. The established path to commercialization taken by Novartis will provide considerable motivation for cell therapy scientists and development companies as well as investors looking to benefit from the ongoing success in the industry.”

The Novartis CAR-T approval is also significant progress for patients and patient communities of the diseases that late-stage clinical CAR-T therapies are targeting. This includes not only patients with late stage acute lymphoblastic leukemia (ALL) that Novartis CTL019 (tisagenlecleucel) is targeting. Kite Therapeutics has also filed a BLA with the FDA for a CD19 CAR, in relapsed/refractory Non Hodgkin Lymphoma, and has filed a Marketing Authorization Application in Europe. Novartis will follow with an application in relapsed/refractory Non Hodgkin Lymphoma in the US and Europe this autumn.

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“The FDA approval by Novartis is a critical validation of the significant international efforts into the CAR-T field. This comes 25 years after the creation of ISCT. Reaching this step will provide significant incentive and motivation for all 1,300 ISCT members working right across the entire cell and gene therapy sector from academics, industry and regulators around the world,” said Catherine Bollard, President, ISCT and invited for the FDA Oncologic Drugs Advisory Committee Novartis CAR-T decision as an ‘invited subject matter expert’. “The Novartis approval is also a momentous milestone in the path for cell therapies reaching patients – the final objective of those of us involved in developing therapies. The approval for CTL019 (tisagenlecleucel) will increase the chance of, and potentially reduce the time for approval for treatments for other critical or fatal diseases with no alternative therapeutic option. ISCT will continue to drive to support all elements of the sector to make sure this happens.”

However, ISCT points out that there is still important work and challenges ahead for the cell therapy industry on effective translation for cost effective therapies to reach patients. Whilst the milestone passed by Novartis is very significant, there are still many more future challenges to overcome for CAR-Ts and cell and gene therapy treatment to become routine options for patient treatment. Commercialisation, market access, patient and physician adoption, cost/benefit discussions and reimbursement are still considerable challenges for the entire sector. Manufacturing scale up still continues as a critical challenge. ISCT believes the entire cell therapy sector must continue to closely collaborate to successfully overcome these challenges.

“It is critical to have a realistic outlook and to have a measured response to the Novartis CAR-T approval. There are many important hurdles requiring different solutions for the industry in the short and long term future. It will be several years before cell therapies are widely accessible for patients in more indications beyond leukemia and lymphoma,” said Miguel Forte, MD, PhD, Chief Commercialization Officer, ISCT and Chief Medical Officer, Bone Therapeutics. “Solid tumors will fast become the next challenge for CAR-Ts and other cell and gene lymphocyte targeted immunotherapies. However, each hurdle will provide additional incentive, investment and know-how to progressively overcome the next. Now Novartis has proved it is possible for one therapy to succeed, this proves others will succeed too, and this adds competition to the mix. There is no bigger engine of development than competition.”

About the International Society for Cellular Therapy

Established in 1992, the International Society for Cellular Therapy (ISCT) is a global society of clinicians, regulators, researchers, technologists and industry partners with a shared vision to translate cellular therapy into safe and effective therapies to improve patients’ lives worldwide.

ISCT is the global leader focused on pre-clinical and translational aspects of developing cell-based therapeutics, thereby advancing scientific research into innovative treatments for patients. ISCT offers a unique collaborative environment that addresses three key areas of translation: Academia, Regulatory and Commercialization. Through strong relationships with global regulatory agencies, academic institutions and industry partners, ISCT drives the advancement of research into standard of care.

Comprised of over 1,300 cell therapy experts across five geographic regions and representation from over 50 countries, ISCT members are part of a global community of peers, thought leaders and organizations invested in cell therapy translation. For more information about the society, key initiatives and upcoming meetings, please visit:

- www.celltherapysociety.org
- [@ISCTglobal](https://twitter.com/ISCTglobal)

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