International Society for Cellular Therapy
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ISCT announces support for FDA Regenerative Medicine Policy Framework

Vancouver, Canada, November 20, 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 reaction to the release of the FDA's newly released comprehensive Regenerative Medicine Policy Framework.

The new FDA framework provides additional clarification on the FDA's risk-based regulations to define drugs, devices, and/or biological products in the regenerative medicine and cell and gene therapy industries, including processes for maintaining safety and efficacy. It also provides additional clarification for manufacturers and for fast track approval processes of new therapies. Importantly, the new framework defines future enforcement actions against unproven therapies, treatments and products that raise potential safety and ethical concerns.

ISCT leaders, on behalf of the more than 1,500 members worldwide, support continuing clarification and action from the FDA of the regulation of the cell therapy industry. ISCT has worked for years to develop initiatives to deal with potentially harmful unproven cellular therapies across the globe – ISCT references for this from the Presidential Task Force on the Use of Unproven Cellular Therapies are here: http://www.celltherapysociety.org/page/UCT. ISCT initiatives have included advocacy for stronger legislative oversight and improved frameworks with which to regulate the unproven cell therapy industry. As regulatory agencies worldwide review these FDA actions, the ISCT global leadership particularly welcomes the FDA's stronger stance on unproven therapies and the additional clarifications on legislation around regenerative medicine.

The FDA qualifications for fast approvals for gene and cell therapies proving sustained therapeutic benefits and efficacy will also provide considerable incentive to all stakeholders in the sector to innovate new cell and gene therapies. The FDA framework will further support, together with actions from other regulators, additional guidance and support for the cell therapy industry internationally leading to faster delivery of documented benefit to patients.

"ISCT welcomes the efforts of the FDA to provide additional regulatory clarification for all those operating in the cell therapy field. However, it is essential that the FDA is now cautious and measured in its introduction and application of the framework. The FDA needs to balance bringing those operating outside the regulatory pathways to compliance, taking action against those that remain outside the licensing and regulatory frameworks, and continuing to foster the ongoing innovation and considerable potential for the majority of the sector operating within the regulatory frameworks," said Catherine Bollard, President, ISCT. "An over-zealous regulatory

application could increase development and manufacturing bureaucracy and costs and time to market, and delay the validation of products and facilities. Ultimately, though, it is patients that are the customers of cell and gene therapies. They have a fundamental right to expect a scientific and clinical rationale for a proposed treatment or therapy they might receive. 2017 has been a seminal year for the cell and gene therapy industry, with the FDA approval of the first two Chimeric Antigen Receptor (CAR) T cell therapies for leukemia and lymphoma, and a considerable number of therapies to imminently seek their own approvals. However, ISCT is still working across the cell and gene therapy sector to provide resolution to issues such as costs of development and pricing and reimbursement. ISCT in its capacity as the organization representing all stakeholders globally across the cell therapy field, will be happy to provide assistance to the FDA, to review progress as the framework is implemented."

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 1,500 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**
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