ISCT International Society for Cellular Therapy
TELEGRAFT
MEMBER NEWSLETTER

North America Legal and Regulatory Affairs Watchdog Update

REGULATORY WATCHDOG

FDA

Some BIG BIG NEWS this Month!

Novartis gets FDA Green-Light on First US CAR-T/Gene Therapy!

Kymriah (tisagenlecleucel) has now been approved for certain pediatric and young adult patients with a form of acute lymphoblastic leukemia (ALL). Price Tag - $475,000:

https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm574058.htm

After 10+ years, FDA Finally Clamp Down on Predatory Stem Cell Clinics!

Statement from FDA Commissioner Scott Gottlieb, M.D. on the FDA's new policy steps and enforcement efforts to ensure proper oversight of stem cell therapies and regenerative medicine:

https://www.fda.gov/newsevents/newsroom/pressannouncements/ucm573443.htm

Warning Letter issued to US Stem Cell Clinic of Sunrise, Florida:

"Stem cell clinics that mislead vulnerable patients into believing they are being given safe, effective treatments that are in full compliance with the law are dangerously exploiting consumers and putting their health at risk,"

https://www.fda.gov/newsevents/newsroom/pressannouncements/ucm573431.htm

See full letter here.

FDA Approves First Ever Treatment for Chronic GvHD

ISCT International Society for Cellular Therapy
TELEGRAFT
MEMBER NEWSLETTER

The U.S. Food and Drug Administration today expanded the approval of Imbruvica (ibrutinib) for the treatment of adult patients with chronic graft versus host disease (cGVHD) after failure of one or more treatments. This is the first FDA-approved therapy for the treatment of cGVHD.

https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm569710.htm?source=govdelivery&utm_medium=email&utm_source=govdelivery

New Approval: Bone Marrow Collection Kit with Flexible Pre-Filter and Inline Filters and Bone Marrow Collection Stand

Fresenius Kabi AG was granted approval for its new substantially equivalent 510(k) device. Full details can be found here:

https://www.fda.gov/BiologicsBloodVaccines/BloodBloodProducts/ApprovedProducts/SubstantiallyEquivalent510kDeviceInformation/ucm568933.htm

CBER Issues Guidance for Biological Products that may be Impacted by Severe Weather Conditions

CBER is providing interested persons with information concerning the storage and use of temperature-sensitive biological products that have been involved in a temporary electrical power failure or flood conditions. For full details, see below:

https://www.fda.gov/BiologicsBloodVaccines/SafetyAvailability/ProductSecurity/ucm147243.htm

Health Canada

Notice of Guidance Document Publication - Use of Certificates of Suitability as supporting information in Drug Submissions

A final version of the guidance document "Use of Certificates of Suitability as supporting information in Drug Submissions" is now available. This results from comments and suggestions received from the consultation between January 31, 2017 and May 1, 2017:

https://www.canada.ca/en/health-canada/services/drugs-health-products/drug-products/announcements/notice-use-certificates-suitability-supporting-information-drug-submissions.html

ISCT International Society for Cellular Therapy
TELEGRAFT
MEMBER NEWSLETTER

Validation rules for regulatory transactions provided to Health Canada in the "non-eCTD electronic-only" format

Health Canada is publishing a set of validation rules for regulatory activities in the "non-eCTD electronic-only" format. These rules build on the information provided in the Guidance Document: "Preparation of Regulatory Activities in "Non-eCTD Electronic-Only" format and will assist sponsors in preparing their regulatory transactions for filing to Health Canada:

https://www.canada.ca/en/health-canada/services/drugs-health-products/drug-products/applications-submissions/guidance-documents/common-technical-document/notice-validation-rules-non-ectd-electronic-only-format.html

Notice - The Regulatory Enrolment Process (REP) Functional Pilot for eCTD Format - Stage III (Sept 2017 – Feb 2018)

In order to gain further experience with the regulatory enrolment process (REP) and to provide an opportunity for additional sponsors to begin using REP, Health Canada is announcing Stage III of the REP Functional Pilot for regulatory activities in electronic common technical document (eCTD) format. This is meant to increase the number of enrolments and transactions received from existing sponsors and provide an opportunity for additional companies to get familiarized with REP. It will assist in assessing the feasibility of enrolling companies, dossiers, regulatory activities and regulatory transactions using REP. Once fully implemented, the REP will reengineer existing administrative processes to take advantage of the tools and capabilities of an electronic processing and review environment. It will introduce a consistent approach to collecting high quality metadata across multiple regulatory activity types:

https://www.canada.ca/en/health-canada/services/drugs-health-products/drug-products/announcements/notice-regulatory-enrolment-process-functional-pilot-stage-iii.html