US FDA and International Regulatory Efforts in Cellular and Gene Therapies

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FDA’s Goals for International Harmonization

- To safeguard global public health
- To assure that consumer protection standards and requirements are met
- To facilitate the availability of safe and effective products
- To develop and utilize product standards and other requirements more effectively
- To minimize or eliminate inconsistent standards internationally
International Engagements for Cell and/or Gene Therapies

• Regulatory exchanges
  http://www.fda.gov/InternationalPrograms/Agreements/ConfidentialityCommitments/default.htm
• FDA-EMA-Health Canada ATMP “Cluster”
• Regulators Forum (ICH)
• Asian-Pacific Economic Communities Life Sciences Innovation Forum (APEC/LSIF)
International Engagements for Cell and/or Gene Therapies (continued)

• Hosting of international regulatory colleagues
  – EMA
  – Japan Pharmaceutical and Medical Device Agency (PMDA)
  – Singapore Health Sciences Authority
  – Swissmedic

• Responses to foreign regulatory inquiries
FDA-EMA-HC ATMP “Cluster”

• Established in 2008 with FDA and EMA, HC joined in 2012
• Regular teleconferences to share thinking on regulatory approaches, both general and specific issues
• Information sharing on draft documents
• Engage reciprocally in workshops and advisory committees, working parties
Regulators Forum (ICH)

• Purpose: to provide an environment in which regulatory and scientific expertise can be shared among regulatory authorities in order to enhance the availability of safe and effective products in the global market

• Meetings of the RF are held in association with ICH biannual meetings
Members of Regulators Forum

- ICH members: US FDA, EMA, and Japan PMDA/MHLW
- ICH observers: Canada, European Free Trade Association (EFTA), WHO
- Regional harmonization initiatives: Asia-Pacific Economic Cooperation (APEC), Association of the Southeast Asian Nations (ASEAN), Southern African Development Community (SADC), Gulf Cooperation Council (GCC), Pan-American Network for Drug Regulatory Harmonization (PANDRH)
- Individual drug regulatory authorities from the following countries: Australia, Brazil, China, Chinese Taipei, India, Republic of Korea, Russia, and Singapore
Formation of a Regulators Forum Cell Therapy Group

• In 2010, FDA proposed that the Regulators Forum undertake a preliminary assessment of potential areas of harmonization for cell therapy products

• The Forum participants agreed that a brainstorming group would be put in place to discuss potential areas for harmonization as well as possible approaches to harmonization
Why Consider Harmonization for Cell Therapy Products?

- Cell therapy is an emerging product class posing unique regulatory challenges
- Regulatory frameworks are in different states of maturity internationally
- Limited experience in reviewing marketing applications for cell therapy products
- ICH & non-ICH guidelines not directly applicable to CT products
- Harmonization of technical requirements would be a useful tool to strengthen the safe and effective use of cell-based products
Are We Ready for Harmonization?

• “Harmonization” is typically interpreted as the production of consensus guidelines

• However, “harmonization” can also refer to a convergence of regulatory perspectives that informs the independent development of national guidelines and regulations

• The term “regulatory convergence” can be used to describe interactions that generate a shared regulatory perspective but do not have the explicit goal of production of a consensus guideline
Regulators Forum Cell Therapy Group (RFCTG)

**Goal:** Information sharing leading to convergence/harmonization of regulatory approaches for cell therapy products

- 6 teleconferences since 2010
- Activities:
  - Compiled an overview of regulatory frameworks from participating countries
  - Compiled a list of scientific and regulatory terminology
  - Identified potential quality and clinical work topics
Participants in RFCTG

- Australia-- Therapeutic Goods Administration (TGA)
- Brazil-- ANVISA
- Canada-- Health Canada
- Chinese Taipei/Taiwan-- Taiwan Food and Drug Administration (TFDA)/Center for Drug Evaluation (CDE)
- European Union-- European Medicines Agency (EMA)
- Japan-- Pharmaceuticals and Medical Devices Agency (PMDA) and Ministry of Health, Labor, and Welfare (MHLW)
- Singapore-- Health Sciences Authority (HSA)
- South Korea-- Korea Food and Drug Administration (KFDA)
- Switzerland-- Swissmedic
- U.S.-- Food and Drug Administration (FDA)
- World Health Organization (WHO), Pan American Health Organization (PAHO)
Regulators Forum Gene Therapy Group (RFGTG)

- **Goal:** share regulatory information among international regulatory authorities including those from emerging economies; continue activities initiated by ICH GTDG

- Initial meeting in Fall 2012

- Prior ICH Gene Therapy Discussion Group, 2000-2011
  - Held workshops
  - Considerations documents published
Participants in RFGTG

- Brazil -- ANVISA
- Canada -- Health Canada
- European Union -- European Medicines Agency (EMA)
- Japan -- Pharmaceuticals and Medical Devices Agency (PMDA) and Ministry of Health, Labor, and Welfare (MHLW)
- India -- National Institute of Biologics
- Singapore -- Health Sciences Authority (HSA)
- South Korea -- Korea Food and Drug Administration (KFDA)
- Switzerland -- Swissmedic
- U.S. -- Food and Drug Administration (FDA)
Asia-Pacific Economic Cooperation (APEC)

• Established in 1989 to promote and facilitate trade
• Members: Australia, Brunei Darussalam, Canada, Chile, China, Chinese Taipei, Indonesia, Japan, Korea, Malaysia, Mexico, New Zealand, Papua New Guinea, Peru, Philippines, Russia, Singapore, Thailand, United States, Vietnam
Life Sciences Innovation Forum (LSIF)

- Established in 2002
- Tripartite forum of government, industry, and academia
- Purpose: Create a policy environment for life sciences innovation
- Guiding Principles: transparency, meaningful dialogue with stakeholders, due process for successful implementation
- Activities: workshops and capacity building
APEC/LSIF Regulatory Harmonization Steering Committee

- Supports activities of the APEC/LSIF
- Primary objectives are to:
  - identify international standards and guidelines to propose to APEC economies
  - facilitate implementation of standards and guidelines through education and workshop support
New Priority Work Area for APEC/LSIF/RHSC

• New Priority Work Area accepted in 2012 to “Promote Regulatory Convergence for the Regulation of Cell and Tissue-Based Therapies”
• Short-term goals are to establish a harmonized understanding of cell and tissue-based therapies and to establish training programs
• Long-term goal is to stimulate prospective convergence of technical requirements
• Next step is the development of a Strategic Roadmap
  – Input from team of interested regulators led by Singapore HSA
  – Will outline the project scope, roles and responsibilities, and expected milestones and deliverables
APEC/LSIF Workshop

- APEC/LSIF Stem Cell QA/QC Workshop July 5-7, 2011
  - Bangkok, Thailand
  - 13 countries participated
  - Goal: To bring together a group of stem cell leaders from corporate, academic and government sectors to discuss and further develop a regulatory framework for QA/QC aspects for stem cell therapy products
  - Information Gathering Opportunities
    - Regulatory landscape for cell therapies
    - Guidance documents in place or under development for (stem) cell therapies
    - (Stem) cell therapy products in clinical trials or already licensed
Summary

• US FDA is committed to active participation in international regulatory convergence efforts for cellular and gene therapy products, as part of our mission to facilitate the development of safe and effective medical products in the US and worldwide.

• Cell Therapy Group and Gene Therapy Group of the Regulators Forum and APEC priority work area on Cell and Tissue-Based Therapies are examples of the efforts of international regulatory authorities to work toward regulatory convergence in new areas.

• These efforts are currently in early stages, with initial focus on:
  – gaining a common understanding of the similarities and differences in regulations and policies for these products in different countries/regions
  – identifying common needs and interests
OCTGT Contact Information

• Regulatory Questions: Contact the Regulatory Management Staff in OCTGT at CBEROCTGTRMS@fda.hhs.gov or Lori.Tull@fda.hhs.gov or by calling (301) 827-6536

• OCTGT Learn Webinar Series: http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/ucm232821.htm
Public Access to CBER

CBER website:
http://www.fda.gov/BiologicsBloodVaccines/default.htm

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