Society for Immunotherapy of Cancer

Early Career Scientists Professional Development Session

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Director, Division of Cellular and Gene Therapies (DCGT)

FDA, CBER

Date: October 24, 2012
Time: 1:30 PM to 5:30 PM
Location: Bethesda, MD

Thank you

OCTGT Contact Information

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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

Research Assessment/Management

- Site visit and CBER Advisory Committee recommendations
- Promotion and Conversion Evaluation (PCE) Committee review
- Regulatory workload and quality
- Publications (including guidance documents, research articles and regulatory articles)
- Success in securing external funding

Responsibilities of PIs

Product review

INDs, IDEs, PMAs, 510(k)s, HDEs, licenses, master files, inspections

- regulatory mentoring by branch chiefs

Policy development

working groups, guidance developments, advisory committees

Outreach

presubmittal advice, scientific and regulatory talks, refereeing and editing for journals, chairing sessions at scientific conferences, collaborations relevant to the regulatory science

Research

lab management and scientific leadership, training/mentoring/supervising, publishing papers, grant writing, leveraging/collaboration

Regulatory Reviewer Responsibilities

- Perform regulatory review of INDs, IDEs, BLA, 510(k), PMA and MFs.
- Participate in the development of regulatory policy
- Participate in the development of regulatory guidance documents
- Interact with stakeholders and give regulatory talks at professional meetings and academic institutions
- Collaborate with researcher-reviewers in regulatory science related research projects
- Contribute to and author regulatory review articles published in peer-reviewed and invited journals and books
- Help solve problems to advance cellular, gene therapy, cancer vaccines, immunotherapy, tissue engineered and other products to the market place

Researcher Reviewer Model

- Cellular, tissue engineering, and gene therapies evolve rapidly and continually present new regulatory challenges
- These novel products raise extraordinarily complex issues
- DCGT seeks to foster a cadre of Researcher Reviewer scientists who :
  - perform regulatory review and identify Critical Path research needs to enhance and promote product development and patient safety
  - perform research in key areas to support the FDA mission and help sponsors solve product development problems to advance cellular, gene therapy and other products to the market place

FDA Commissioner's Fellowship Program

- For healthcare professionals, scientists, and engineers. A two-year Fellowship Program, where they will receive regulatory science training and the chance to conduct cutting-edge research on targeted scientific or regulatory issues under the mentorship of an FDA senior scientist.
- Eligibility: A Doctoral level degree (M.D., D.O., D.V.M., D.D.S., D.P.M., Pharm.D., or Ph.D.); however, applicants with a Bachelor's or Master's degree in an engineering discipline will also be considered.
- December through March - Applications accepted. Late June/July – Interviews. July through August – applicants notified. October – Program Start Date

http://www.fda.gov/AboutFDA/WorkingatFDA/FellowshipInternshipGraduateFacultyPrograms/CommissionersFellowshipProgram/default.htm

Additional Fellowships

- **Inter Agency Oncology Task Force (IOTF) Fellowship (Established May 2003)**
  - The FDA and the NCI joint fellowship programs to provide training in product, preclinical, and clinical research and research-related regulatory review.
  - Four types of fellowships
    - Clinical Oncology Product Research/Review for Oncology Fellows
    - Clinical Oncology Product Research/Review for Board Certified (BC) Oncologist
    - Oncology Product Research/Review for Fellows
    - Cancer Prevention Fellows
  - Jan – May- Sep to start program by July – October and July next year, depending upon program

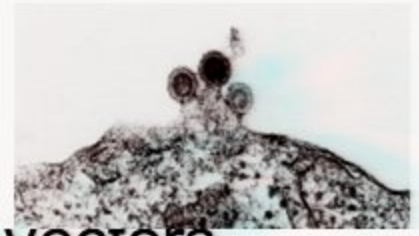
http://biospecimens.cancer.gov/relatedinitiatives/overview/iotf.asp

Types of FDA Positions

- Regulatory Review Scientists
- Regulatory Project Manager/Consumer Safety Officer
- Medical officers
- Researcher Reviewers - Principle investigators (PIs) – tenured or tenure track (Senior staff Fellow or Visiting Scientists)
- Staff Scientists – tenured researcher reviewers supporting PIs program: do both review and research
- Staff Fellows or Visiting Associates: do both review and research work
- Technicians: do primarily research, lab manager, some do limited review work
- Postdoctoral fellows funded as ORISE: do primarily research

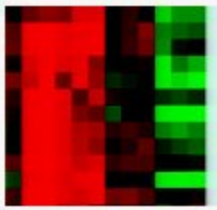
Current DCGT Research Areas

- Virology
  - Retroviruses, adenovirus



- Immunology
  - Immune responses to viral and plasmid vectors,
  - autoimmunity and immune regulation
- Cell and developmental biology
  - Control of differentiation in animal models:
  - cell fate and survival, stem cell biology

- Cancer biology
  - Molecular biomarkers, cancer vaccines, animal models



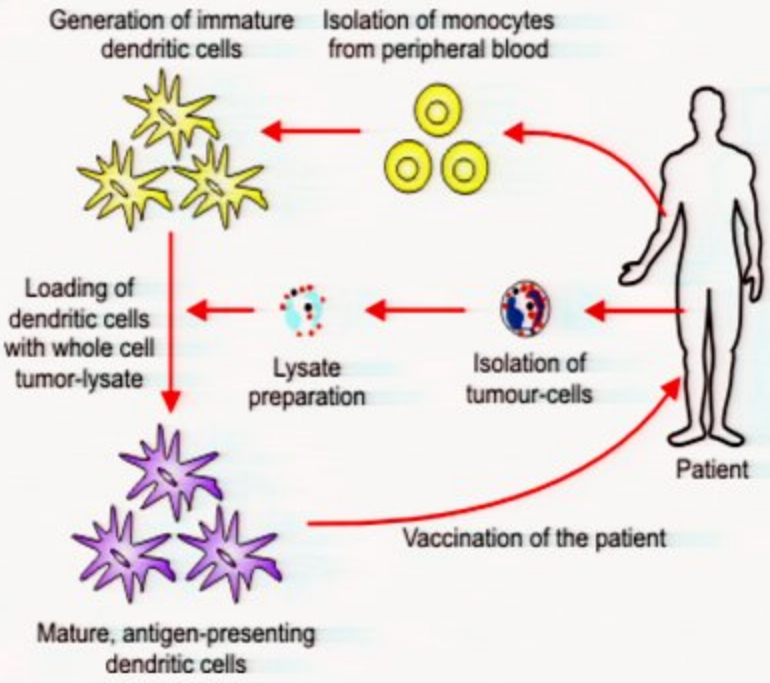
- Biotechnology
  - Genomics, flow cytometry, proteomics, transgenics
- Microbiology of tissue safety
  - Pyrosequencing and whole genome sequencing

CBER research: Stay ahead of the curve as products and technologies evolve

Personalized medicine, stem-cell derived products, recombinant vaccines, engineered tissues, etc.

Products and regulatory paradigms are evolving, not standing still.

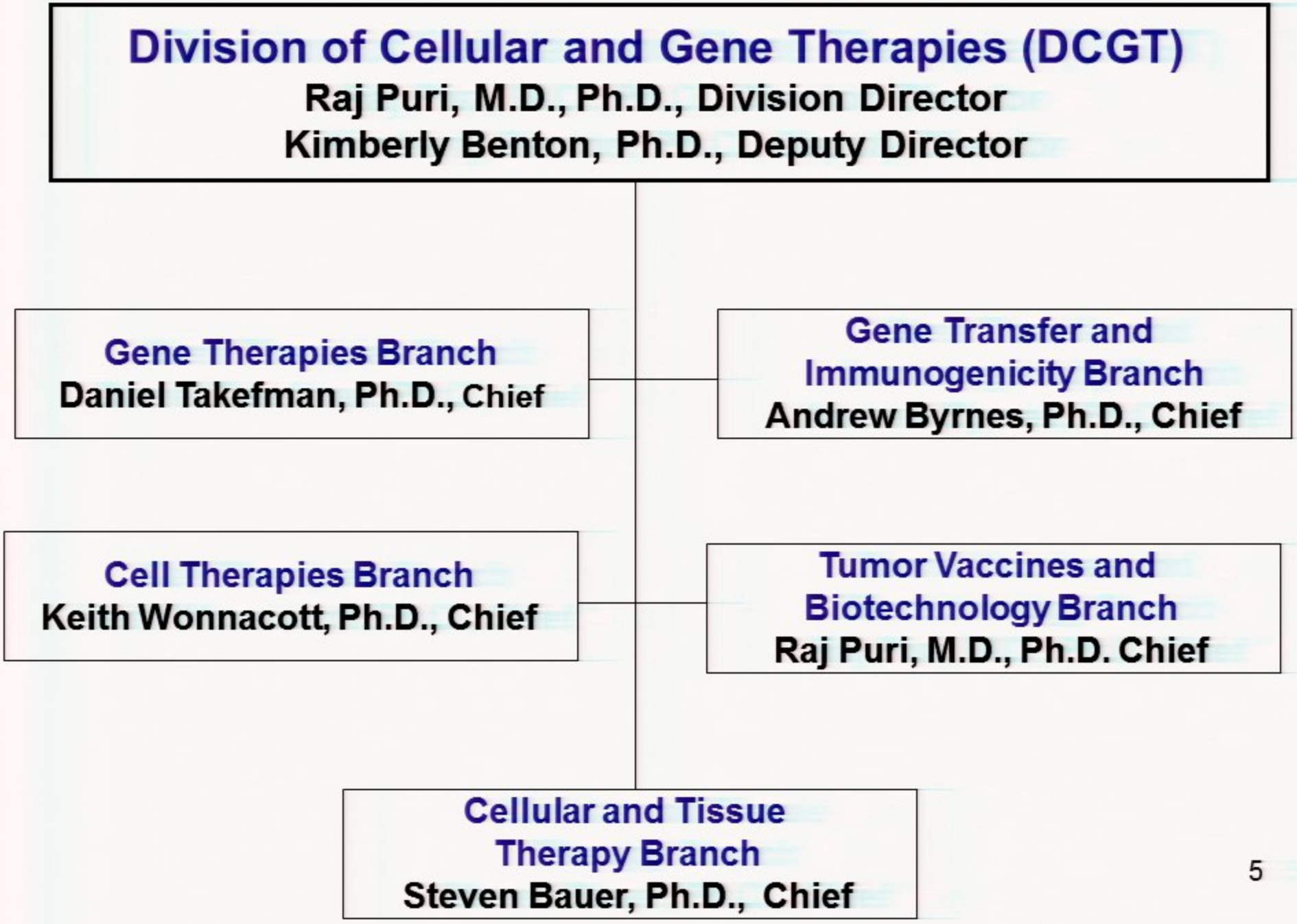
Cutting-edge research at CBER helps prepare the way for anticipated products and develop appropriate policy.



Manufacturing a dendritic cell cancer vaccine

Regulation of biological products

Regulation based on science, law, and public health impact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Raj Puri, M.D., Ph.D. Chief
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CBER Office of Cellular, Tissue, and Gene Therapies (OCTGT)

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Suzanne Epstein, Ph.D. Associate Director of Research
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Division of Human Tissues

Ellen Lazarus, M.D., Director

Division of Clinical Evaluation and Pharmacology/Toxicology

Wilson Bryan, M.D., Director

OCTGT Regulated Products

- Cellular therapies
- Umbilical Cord Blood
- Tumor vaccines and immunotherapy
- Gene therapies
- Tissue and tissue based products
- Xenotransplantation products
- Combination products
- Devices used for cells/tissues
- Donor screening tests (for use with cadaveric blood samples)

CBER/ FDA regulates biological products

Science-based regulation, research an integral part

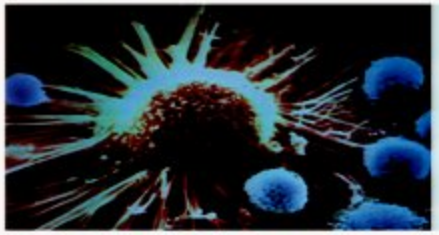
Blood Derivatives

Vaccines

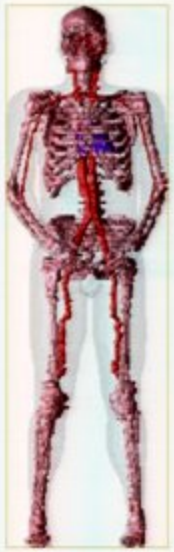
Blood Components

Allergenics

Whole Blood



Somatic Cell & Gene Therapy



Devices

Devices

Tissues

Xenotransplantation