

Regenerative Cell Therapies:

Making Safe and Effective Therapies Available to Patients

Congressional Briefing

Hart Senate Office Building, Room 902

September 19, 2019

12pm – 1pm ET

 Alliance for Cell Therapy Now



REGENERATIVE
MEDICINE
FOUNDATION



About Alliance for Cell Therapy Now

- Independent, non-profit organization devoted to advancing the availability of safe and effective cell therapies for patients in need.
- We bring together experts and stakeholders—working in collaboration with other organizations that have a similar mission—to gain consensus on and advocate for sound policies that improve the development, manufacturing, delivery, and improvement of safe and effective regenerative therapies.

Where We Are

- Regenerative cell therapies represent next generation of groundbreaking treatments
- Several well-designed, FDA-approved clinical trials are being conducted
- At the same time, a small number of clinics are causing patient harm or making questionable claims, casting negative light on this emerging field
- 21st Century Cures Act laid the groundwork for these promising therapies, extending FDA expedited programs and providing funds for NIH research

What's Needed

- Development, consensus, and adoption of standards to support improvements in development, manufacturing, and delivery
- Launch of outcomes database to advance the science, drive improvements in manufacturing and delivery, and inform regulatory evaluation and payment, as well as clinical and patient decision-making
- Workforce development initiatives to build capacity and prepare the skilled, technical workforce for this emerging field.
- Additional funding support for research at the NIH.
- Increased capacity at the FDA to support both enforcement efforts and strategies to support academic and research institutions, as well as innovators, who need assistance in navigating FDA regulatory requirements.



Thank You to our Collaborators for This Event



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We are About Collaboration

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International Federation for Adipose Therapeutics and Science (IFATS)
- Krishnendu Roy, Ph.D.
Georgia Institute of Technology
- Fred Sanfilippo, MD, PhD
Emory University and The Marcus Foundation
- Bernard Siegel, JD, Co-Chair
Regenerative Medicine Foundation
- Robin L. Smith, MD, MBA
Stem for Life and Cura Foundation





The Cura Foundation is a nonsectarian, nonpartisan, public and tax-exempt organization under Section 501(c)(3) of the Internal Revenue Code. Cura's mission is to improve human health, streamline health care delivery and optimize access globally in order to prevent and vanquish disease, reduce human suffering and increase quality of life.

At Cura, we **#UNITETOCURE.**

The foundation aims to fund emerging technologies to prevent diseases from occurring and accelerate cures for those diseases that cannot be prevented. By utilizing predictive analytics and big data to help determine the correct treatments for individuals, we can use resources more efficiently and with technology, we can improve access to care and prevent diseases earlier in the process. We foster collaboration between scientists, physicians, regulatory agencies, ministers of health, patient advocates, physicians, ethicists, philanthropists, leaders of faith, government officials and industry personnel.

The foundation also educates the next generation of thought leaders about the innovation in cell therapy, personalized medicine and the importance of interfacing with data to improve clinical outcomes and optimize health care spending.

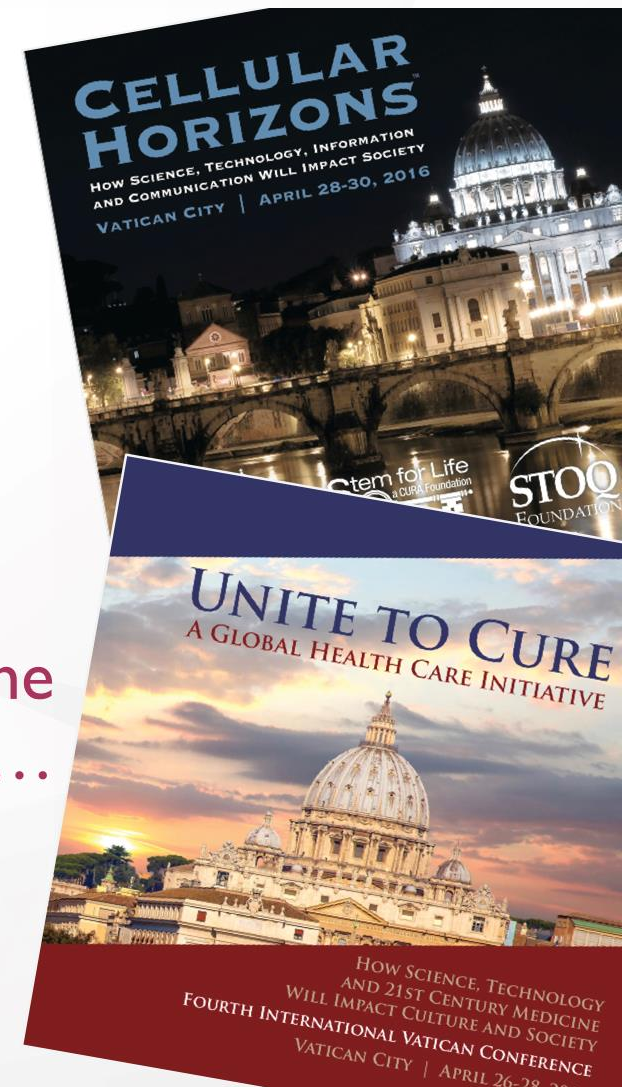
Stem For Life and Cura coordinate their efforts to unite to cure by accelerating interest in the development of cellular treatments, unlocking the body's natural healing and repair mechanisms to vanquish disease and restore damaged tissue and organs.

Together they look for ways to speed clinical development and utilize big data and algorithmic learning to advance personalized medicines so that patients can receive the best possible treatment for their individual diseases as quickly as possible rationalizing health care dollar spent while improving clinical outcomes.



The Vatican Conferences were a huge success and have reached over 3.5 billion people!

We were joined by some very special individuals...





Francis S. Collins, MD, PhD
 16th Director, U.S. NIH
 2018 Pontifical Key Scientific Awardee



**His Holiness
 Pope Francis**



Joseph R. Biden, Jr.
 47th U.S. Vice President



Andrew von Eschenbach, MD
 Samaritan Health Initiatives,
 Former Commissioner, U.S. FDA



Robert M. Califf, MD
 Duke Health and Verily
 Former U.S. FDA Commissioner



James P. Allison, PhD
 University of Texas
 MD Anderson Cancer Center



William Frist, MD
 Physician, Former U.S.
 Senate Majority Leader



Tommy G. Thompson
 42nd Governor of Wisconsin,
 Former Secretary, U.S. HHS

Collaborating on Advancement of Outcomes Database

- AABB
- Adam Susser Foundation
- Alliance for Cell Therapy Now
- American Academy for Regenerative Medicine
- Amnion Foundation
- California Association of Hepatitis C Task Force
- Center for International Blood and Marrow Transplant Research (CIBMTR) at the Medical College of Wisconsin
- Cord Blood Association
- The Cure Alliance
- Duke University School of Medicine
- Institute for Stem Cell and Regenerative Medicine, Oakland University and Beaumont Health System
- International Association of Hepatitis Task Forces
- International Federation of Adipose Therapeutics and Science (IFATS)
- International Perinatal Stem Cell Society
- The Loving Mind Institute
- Marcus Center for Therapeutic Cell Characterization and Manufacturing at Georgia Tech
- Marcus Foundation
- Mayo Clinic
- Missouri Cures Education Foundation
- National Marrow Donor Program (NMDP): Be the Match
- Nova Southeastern University Cell Therapy Institute
- NSF Engineering Research Center for Cell Manufacturing Technologies (CMaT)
- Parker H. Petit Institute for Bioengineering and Bioscience at Georgia Institute of Technology
- Regenerative Medicine Manufacturing Society
- Regenerative Outcomes Foundation
- Regenerative Medicine Foundation
- Sabrina Cohen Foundation (SCI and disability)
- Sanford Health
- Stand Among Friends
- Stem for Life, a Cura Foundation
- Student Society for Stem Cell Research
- Texas Center for Cell Therapy and Research, LLC
- Tulane Center for Stem Cell Research and Regenerative Medicine
- University of Florida Center for Regenerative Medicine
- University of Miami, Interdisciplinary Stem Cell Institute at the Miller School of Medicine
- Wake Forest Institute for Regenerative Medicine



Overview of Today's Briefing

- **Panel 1: Regenerative Cell Therapy Research: Current Clinical Trials and the Promise for Patients**
 - Anthony Atala, MD, G. Link Professor, and Director, Wake Forest Institute for Regenerative Medicine, Wake Forest University School of Medicine
 - David Pearce, PhD, President of Innovation and Research, Sanford Health; Scientist, Pediatrics and Rare Diseases Group; Professor, Department of Pediatrics, Sanford School of Medicine of the University of South Dakota
 - Joanne Kurtzberg, MD, Jerome Harris Distinguished Professor of Pediatrics; Professor of Pathology; Director, Marcus Center for Cellular Cures; Director, Pediatric Blood and Marrow Transplant Program; Director, Carolinas Cord Blood Bank; Co-Director, Stem Cell Transplant Laboratory; Duke University Medical Center
 - Moderated by Fred Sanfilippo, MD, PhD, Professor, Health Policy and Management, Rollins School of Public Health, Emory University; Director, Emory-Georgia Tech Healthcare Innovation Program; Medical Director, The Marcus Foundation



Overview of Today's Briefing

- **Panel 2: Cell Characterization and Scalable Manufacturing: Assuring Quality Control and Improving Access for Cell Therapies**
 - Krishnendu Roy, PhD, Robert A. Milton Chair; Director, NSF Engineering Research Center for Cell Manufacturing Technologies; Director, Marcus Center for Cell-Therapy Characterization and Manufacturing; Technical Lead, National Cell Manufacturing Consortium; Director, Center for ImmunoEngineering, Georgia Institute of Technology
 - Moderated by Fred Sanfilippo, MD, PhD, Professor, Health Policy and Management, Rollins School of Public Health, Emory University; Director, Emory-Georgia Tech Healthcare Innovation Program; Medical Director, The Marcus Foundation



Overview of Today's Briefing

- **Panel 3: Protecting Patients Through Rigorous Science and Safety**
 - Bernard Siegel, JD, Executive Director, Regenerative Medicine Foundation
 - Amy Patterson, MD, Chief Science Advisor and Director of Scientific Research Programs, Policy, and Strategic Initiatives, Immediate Office of the Director, National Heart, Lung, and Blood Institute, National Institutes of Health
 - Peter Marks, MD, PhD, Director, Center for Biologic Evaluation and Research, Food and Drug Administration
 - Moderated by Janet Marchibroda, President, Alliance for Cell Therapy Now

