



Alliance for Regenerative Medicine

Michael Werner
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Alliance for Regenerative Medicine



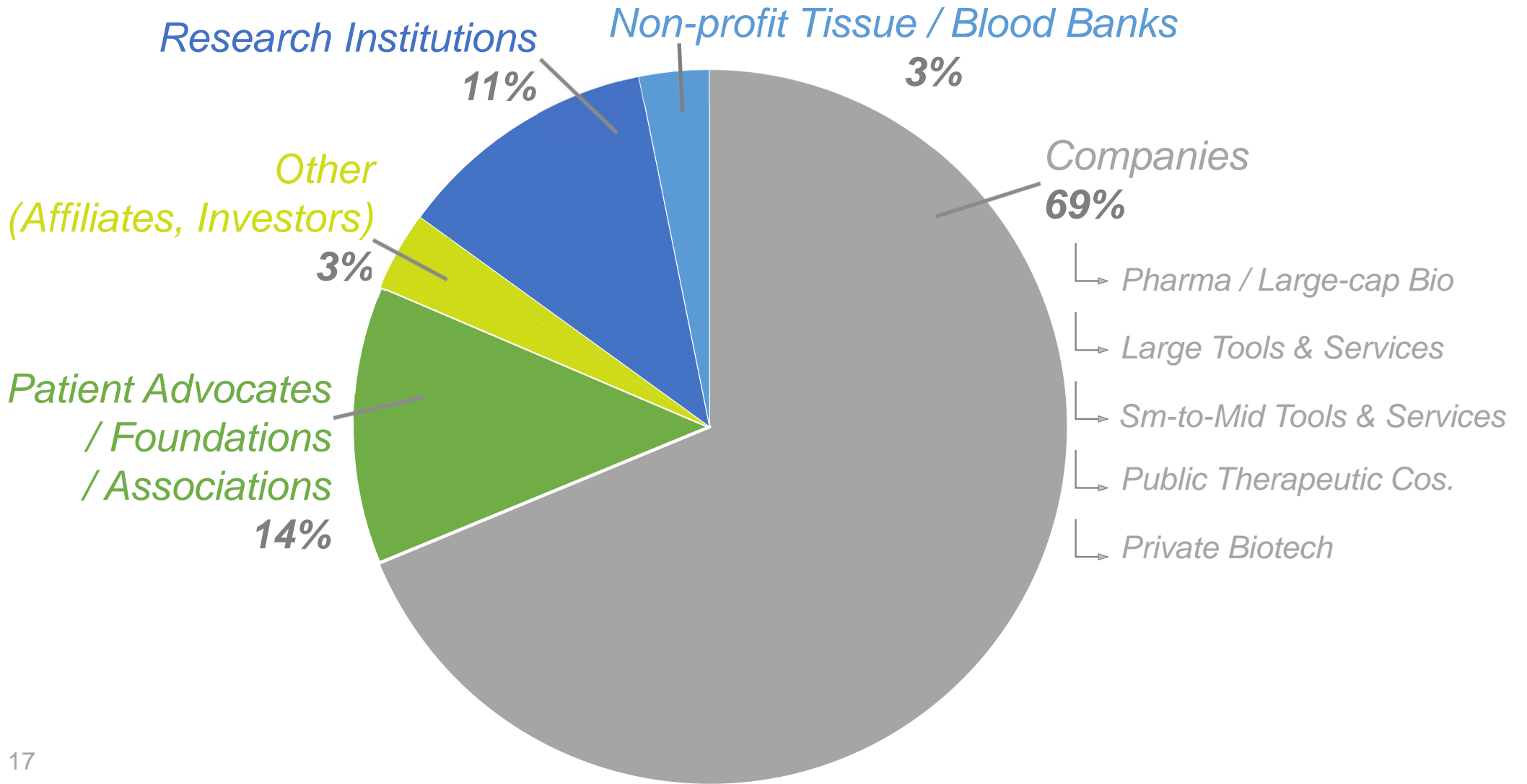
ARM's Focus & Role

As the leading global advocate for the regenerative medicine and advanced therapies sector, ARM enables acceleration of research, development, investment and commercialization of transformational treatments and cures for patients worldwide.

- Drives regulatory, scientific and policy advancement*
- Strives to bring transformative regenerative medicines to patients as soon as possible*
- Maximizes market access for therapeutic developers*
- Enables sustainable access to capital*

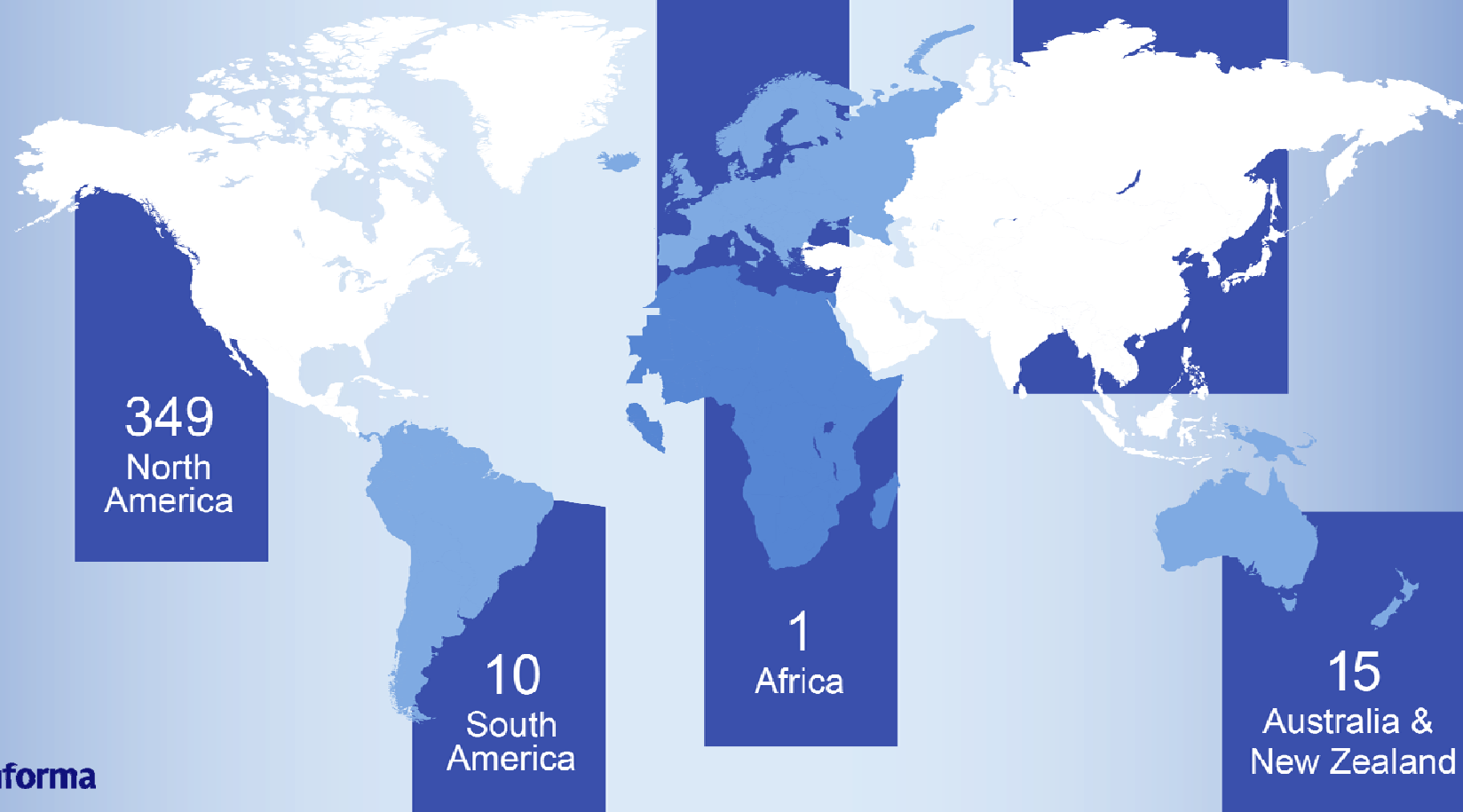
TOTAL ARM MEMBERS = 250+

ARM Membership Composition



672+

Regenerative Medicine Companies Worldwide,
Including Gene and Cell Therapies



Data provided by: **informa**

Clinical Progress: End of 2015



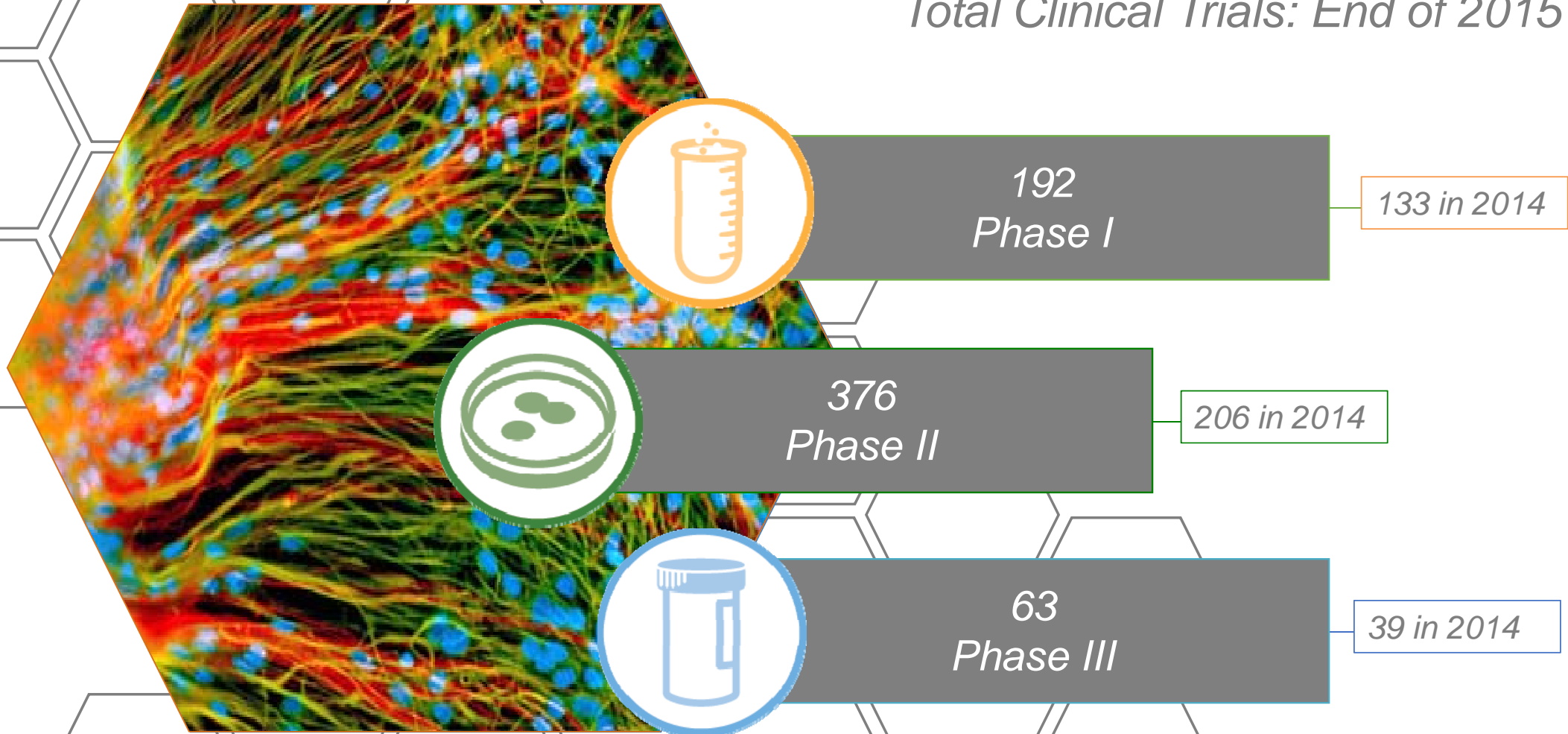
20
Approved RM/AT
Products Worldwide



631
Total Clinical Trials

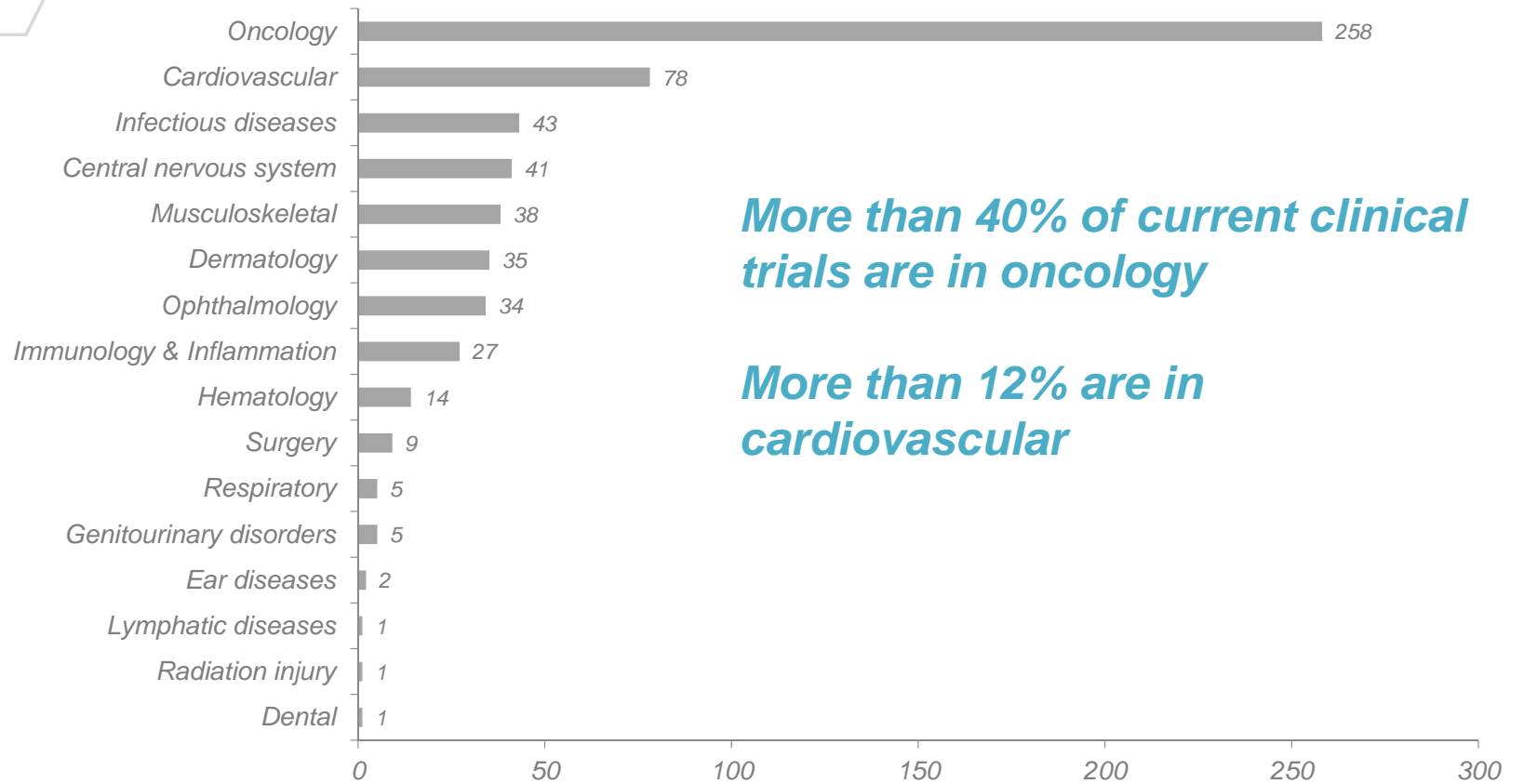
Includes both industry & investigator-initiated clinical trials

Total Clinical Trials: End of 2015



Data provided by: **informa**

Clinical Trials by Therapeutic Category



ARM Data Reports – Quarterly & Annually

ALLIANCE_{for}
Regenerative Medicine
2015

Industry-specific statistics compiled from more than 670 leading gene therapy, cell therapy and other regenerative medicine companies worldwide, including total financings, partnering and dealmaking, clinical trial information, key data events and ARM's strategic priorities for 2016. Available at <http://alliancerm.org/page/arm-data-reports>

Annual Data Report
on gene and cellular therapies and the regenerative medicine sector



Dr. Robert Califf – New FDA Commissioner

- *Has performed clinical trials – respected by academia, industry and agency staff*
- *Believes in strong role for patients in clinical trials and FDA decision-making*

From his testimony:

- *“To advance, we must find common ground with industry and academia on the science without compromising this fundamental role of the FDA.”*
- *“The emergence of consumers and patients as active participants in the process of developing therapies and devising protocols for evaluation is an important theme to improve the relevance of our work...”*

Impact on RM, HCT/Ps, etc. is unclear, although he is big supporter of medical innovation.



FDA Regulatory Activity: Human Cell & Tissue Products (HCT/Ps)

Minimal manipulation, homologous use, etc. are the subject of new draft guidance and FDA enforcement actions

FDA public meeting re: Draft Guidances Relating to the Regulation of Human Cells, Tissues or Cellular or Tissue-Based Products later this year

FDA Enforcement

December 30, 2015: FDA issued a warning letter to Irvine Stem Cell Treatment Center

- *Recovers and processes adipose tissue, a structural tissue, from donors for autologous use.*
- *Firm isolates cellular components from adipose tissue, which are further processed into stromal vascular fraction (SVF) (aka adipose-derived stem cells).*
- *SVF product is administered intravenously (IV), etc. and intended to treat a variety of diseases and conditions (autism, Parkinson's disease, pulmonary fibrosis, chronic obstructive pulmonary disease (COPD), multiple sclerosis (MS), cerebral palsy, and amyotrophic lateral sclerosis (ALS)).*
- *FDA says this is more than minimal manipulation (processing alters the original relevant characteristics of the adipose tissue) and its for a non-homologous use so 351 is required.*



U.S. Congress: 21st Century Cures / Medical Innovation Legislation

- *Passed the House July 10, 2015*
- *Based upon a desire to make the drug discovery and approval process more efficient*
- *Contains some provisions related to FDA review process especially for “breakthrough medical devices”*
- *Senate prospects currently unclear*
- *Some discussion of “conditional approval”*
- *All the issues may be included in user fee discussions and addressed in 2017*

ARM Regulatory & Legislative Focus Areas: 2016

- *Global reimbursement issues – advancing specific proposals to promote coverage, coding and payment policies that facilitate development of and patient access to gene and cellular therapies and other regenerative medicine products.*
 - *Identifying potential policy and legal impediments to coverage and reimbursement*
 - *Conducting formal analysis of payment models to facilitate access and adoption*
 - *Outreach to U.S. CMS, private payers and EU HTA bodies and reimbursement agencies*
- *Formal establishment of a Standards Coordinating Body in RM*
 - *ARM initiative to coordinate development and implementation of RM standards among key stakeholder groups (especially manufacturing, processing, assay development)*
- *Gene editing and related bioethics issues – currently collaborating with NAS on upcoming consensus report on human gene editing and related regulatory and bioethics issues*
- *Participate in FDA process regarding minimal manipulation guidance*
- *International regulatory convergence*
- *PDUFA reauthorization recommendations*

ARM Advocacy: 21st Century Cures / Medical Innovation Legislation

Advocating for ARM's provisions to be included in congressional efforts to streamline the drug development and review process.

This includes:

- *A modified role for the NIH-Recombinant DNA Advisory Committee (RAC) to ensure the oversight of gene therapy clinical trials is streamlined.*
- *Combination products - ARM advocates for reforms to streamline the review process for combination products or other situations when more than one review center at FDA is involved in product evaluation and review.*
- *Potential new pathway to market*
 - *ARM advocates the FDA designate certain regenerative medicine / advanced therapy products as "Qualified Regenerative Medicine Products" (QRMP), intended for serious and life-threatening diseases with currently no available treatment options.*
 - *The FDA would meet with the QRMP sponsors to discuss expedited review options. ARM is working with policymakers on other potential pathways as well.*
- *We oppose efforts to allow products to market without sufficient evidence of safety and efficacy*
- *Standards Coordinating Body*

Standards Coordinating Body

- *ARM Initiative to better coordinate development and implementation of RM standards among key stakeholder groups*
- *Public Private Partnership with NIST via Cooperative Research and Development Agreement (CRADA) – Application to be submitted February 2016*
- *Commitment to join and support Charter Group from:*
 - *Ten leading RM stakeholder organizations: ARM, ISCT, ISSCR, ASGCT, AABB, ESGCT, TERMIS, Cell Therapy Manufacturing Consortium, UK CT Catapult, and CCRM*
 - *Twelve corporate charter members*
- *Initial meeting of the Charter Group scheduled for January 28, 2016*
- *Two to three projects to be recommended for initial work plan*
- *Charter members will consider funding options to sustain SCB*
- *FDA has expressed strong support for initiative*
- *Once established, SCB will be expanded to include other international stakeholder groups*

Looking Ahead

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- *Likely modest changes to FDA interpretation and enforcement of regulations governing HCT/Ps.*
 - *But agency will remain vigilant that if product is more than minimally manipulated or not for homologous use, the developer will need a BLA*
 - *New Standards Coordinating Body in the U.S. for regenerative medicine*
 - *Charter group meeting*
 - *Growing Congressional support*
 - *Likely other modifications to the market pathway*
 - *RM products may benefit but not clear if designed specifically for RM*
 - *Negotiated as part of “user fee” agreements and legislation*
 - *New regulations governing “supply chain” and distribution/importation*
 - *More RM products on the market in the U.S. and around the world!*

ALLIANCE for
Regenerative Medicine

