

Welcome - Mesa Day Two

ARM's Role in the Sector

Janet Lambert, CEO

October 3, 2019



About ARM

- **International advocacy organization**
 - Dedicated to realizing the promise of safe and effective regenerative medicines for patients around the world
 - Cell and gene therapy, tissue engineering
- **340+ members**
 - Small and large companies, non-profit research institutions, patient organizations, and other sector stakeholders
 - Across 25 countries
- **Priorities:**
 - Clear, predictable, and harmonized **regulatory** pathways
 - Enabling market access and value-based **reimbursement** policies
 - Addressing industrialization and **manufacturing** hurdles
 - Conducting key stakeholder outreach, **communication**, and education
 - Facilitating sustainable access to **capital**

Drive Adoption of New Payment Models and Value-based Reimbursement

- Lobby in support of legislation in the U.S.
 - Held 90+ meetings with congressional offices during ARM Fly-In
 - Organized Congressional educational briefings on gene therapy, market access
 - *Key Provisions included in Senate Finance Committee-approved bill*
- Testified at the CMS Town Hall, filed comments, met with CMS seeking improvements in CAR-T coverage and reimbursement
 - *NTAP increased from 50% to 65%*
 - *National Coverage Decision finalized*
 - *CMS will consider CAR-T DRG for FY 2021*
- Analytical Work
 - Summer '19: Status of market access for ATMPs in key European countries, including barriers to and recommendations for timely patient access
 - Fall '19: Roadmap for Managed Care Access, w/NAMCP
 - Fall '19: Potential Cost Saving of Cell And Gene Therapies For The Next Decade



ARM presents Senator Cassidy with the Legislator of the Year Award, 2019

Regulatory Pathways

Clear, Predictable, Effective, Harmonized Regulation of Regenerative Medicines



Dr. Peter Marks, Director of the Center for Biologics Evaluation and Research at US FDA, speaks with attendees at ARM's 2018 Meeting on the Mesa

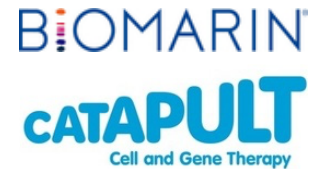


Dr. Guido Rasi, Executive Director of the European Medicines Agency (EMA) at ARM's inaugural 2019 Meeting on the Mediterranean in Barcelona

- Regular engagement with the FDA & EMA; invited to ICMRA meeting in October
- Topics: disease specific guidances, CMC requirements, registries, good clinical practice, trial design, European repository of regulatory requirements, GMO harmonization, ARM's analysis of the discrepancies in US and EU regulations for ATMPs, etc

- Member workshop on comparability in cell & gene therapy development, with USP & global regulators
- *In Vivo* publication on manufacturing challenges in cell and gene therapy in June, and co-hosted a follow-up webinar in September
- Finalizing a comprehensive framework document -- A-Gene -- covering development, manufacture, regulatory submission, and lifecycle of gene therapies
 - Expected release in early 2020
 - Content is currently being drafted for A-Cell, a similar project focusing on cell therapies

A-Gene participants include:



The SCB, co-founded by ARM in 2016, is making major advances in standards that benefit the growth of the entire regenerative medicine field

Connecting the Regenerative Medicine Community to the Standards Development Process

- As an independent, fully-staffed, 501(c)(3) organization, SCB works to **coordinate** standards activities, **engage** experts, and **educate** the regenerative medicine community.
- Drives FDA-funded standards activities (third FDA contract recently executed), collaborates with NIST
- Fifteen standards advancement projects underway
 - Comprehensive landscape reports
 - Industry education and workshops
 - Involved in five ISO standards this year



Connect with an SCB leader here at Mesa19!

FOR MORE INFORMATION VISIT www.standardscoordinatingbody.org OR CONTACT preczek@regenmedscb.org

- Elevated Industry voice in the gene editing international dialogue by releasing a Therapeutic Developers' Statement of Principles on Human Genome Editing
 - Signed by 14 preeminent companies active in developing gene-edited therapies.
 - Audentes, bluebird bio, BlueRock, Caribou Bio, Casebia, CRISPR, Editas, Homology, Intellia, LogicBio, Precision, Sangamo, Tmunity, Pfizer
 - Asserted that the clinical use of germline gene editing is currently inappropriate
- Still accepting signatures from ARM member companies
- Presented at an August 27 meeting of the WHO's expert advisory panel on human genome editing in Geneva; Engaged with NAS gene editing panel



Biotech companies issue first declaration on human gene editing

Industry declares that it will not make DNA changes affecting future generations

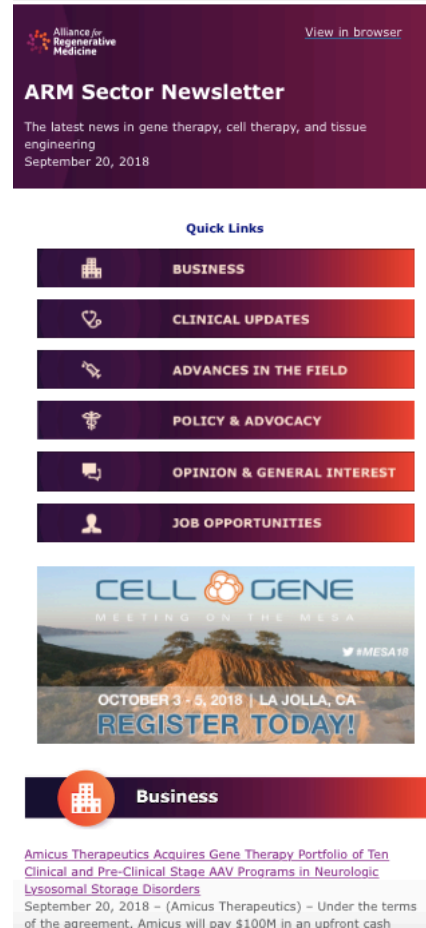
- Sector reports, providing clinical and financial data as well as commentary on the sector
 - Global data
 - Regional data
 - Indication-specific reports
- Sortable, searchable databases:
 - Expedited Approval Designations Database
 - Anticipated Clinical Data Readouts & Trial Milestones
- Available: www.alliancerm.org



Sector Newsletter

Publicly available sector newsletter distributed to nearly 12,000 life science professionals each week.

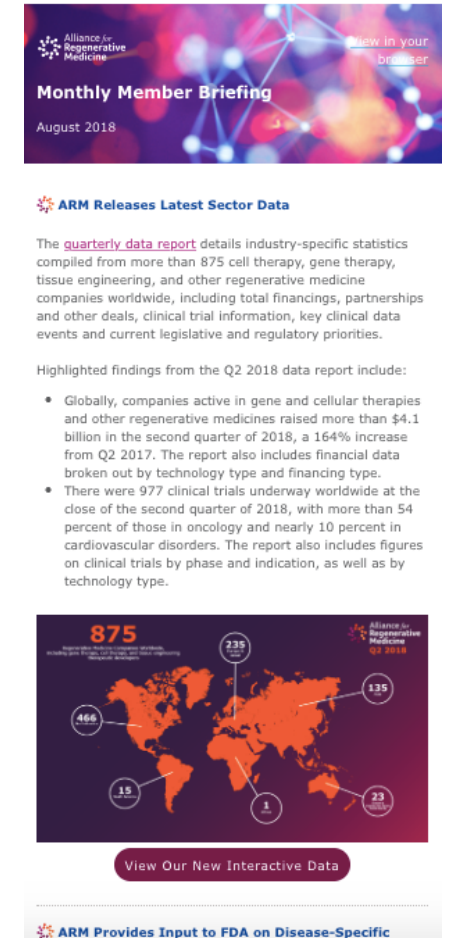
- Recent financings & business deals;
- New clinical data & milestones;
- Scientific advancements;
- Policy news and updates; and
- Job opportunities with ARM members



Member Briefing

Members-only monthly activity briefing, launched 2018.

- Recent and ongoing ARM initiatives;
- Exclusive member memos and publications;
- Opportunities for engagement;
- Notable sector news and events



ARM Foundation for Cell & Gene Medicine

Launched by ARM in 2018

The **ARM Foundation** serves as the **educational and information catalyst** on issues fundamental to making gene and cell therapies, tissue-engineered products and other regenerative medicine treatments available to patients.

By examining, quantifying, clarifying and informing stakeholders of the clinical and societal benefits of these therapies, as well as convening discussions to raise awareness about the sector's progress, challenges and results, the Foundation **accelerates patient access** to safe, efficacious and potentially curative therapies.

Fifteen Minute Overview of the Foundation's Work: Friday 9:00am, BlueRock Ballroom

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For More Information

For more information about ARM or ARM membership:

Visit: www.alliancerm.org

Contact Alyce Osborne, Director of Member Engagement:
AOsborne@alliancerm.org

Follow us on Twitter:
[@alliancerm](https://twitter.com/alliancerm)

Thank You!

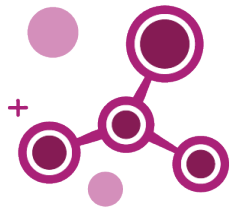
Policy Makers Actively Seeking Guidance in Regenerative Medicine



ARM's Recent Comments, Letters, & Testimony	Purpose	Recipient	Date
Comments on ICER's RFI "Value Assessment Methods for Single or Short-Term Transformative Therapies (SSTs)"	ARM finds that while there are some positive elements in ICER's proposal, overall, the value assessment methods are inadequate to fully reflect the long-term value of these transformative therapies.	ICER	Sept 2019
Comments on the EMA guidance on quality, non-clinical and clinical requirements for investigational ATMPs	ARM recommends to provide additional guidance on some aspects such as non viral in vivo therapeutics and fully synthetic genome editing products, to adapt and accommodate for future improvement of RCV detection methods and in general, to ensure alignment with existing FDA requirements	EMA	July 2019
Comments on the EMA guidance on quality, non-clinical and clinical requirements for genetically modified cells	ARM recommends consistency in language and content with other existing guidelines, promotes international convergence of requirements and commented on some other aspects such as Replication Competent Virus or scope of the guideline	EMA	July 2019
Comments on the Proposed New Standard for Immune Effector Cell Therapy (IECT) Services	ARM believes that the proposed standard for the Certificate of Need Program requiring that any facility to receive both the Commission's approval and a third-party accreditation from the FACT in order to deliver CAR-T therapies to patients in Michigan will be an onerous barrier to access.	MI Dept of Community Health	July 2019
Comments on 'Discussion paper: Use of patient disease registries for regulatory purposes – methodological and operational considerations'	ARM highlighted the need for pan-European RWE infrastructures, multi-stakeholder engagement and international collaboration and made some recommendations on governance to ensure funding and sustainability of registries, for the benefits of patient market access and long-term follow-up.	EMA	June 2019
Public consultation on EMA Regulatory Science to 2025	ARM responded to the EMA questionnaire by identifying and commenting top priorities to address the challenges of ATMP development and foster patient access to treatment.	EMA	June 2019
Comments on the IPPS for Fiscal Year 2020	ARM suggests that CMS adjust its CAR T policies for the NTAP to ensure the appropriate reimbursement of these innovative therapies. ARM's recommendations include a CAR-T specific MS-DRG and recognizing certain FDA approval designations as dispositive for newness and substantial clinical improvement	CMS	June 2019

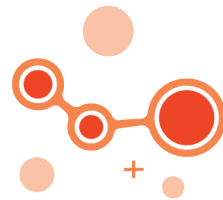
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Comments on the Proposed Rule on the Removal of Safe Harbor Protection for Rebates Involving Prescription Pharmaceuticals	While ARM is supportive of the OIG's efforts to reexamine the applicable regulatory safe harbors currently in place under the anti-kickback statute, we are concerned that the proposed change would serve to also exclude arrangements involving the application of price concessions based on value.	HHS OIG	April 2019
Comments on FDA's 'Rare Diseases: Common Issues in Drug Development; Draft Guidance for Industry'	ARM commends the FDA for the revision of the draft guidance on common issues in rare disease drug development. However, we request the Agency to consider how the guidance can leverage even more comprehensively the flexibility afforded by the FDA in the context of drug development in rare diseases.	FDA	April 2019
Comments on proposed National Coverage Decision (NCD) for Chimeric Antigen Receptor (CAR) T-cell Therapy	ARM asks CMS to ensure Medicare patients who can benefit from CAR T therapies are covered under the NCD, allow CAR T to be administered in certified, trained, experienced facilities and not limit access to hospitals, and certify a registry to ensure continued access to CAR T by Medicare beneficiaries.	CMS	March 2019
Comments on ICER's RFI: Evaluation of Potentially Curative Treatments and for Translating the Results of Cost-Effectiveness Analyses into Recommendations for Value-Based Price Benchmarks	It is ARM's view that traditional HTA frameworks in both U.S. and Europe are not flexible enough to accommodate potential cures and do not allow the ability to capture the full product value due to issues including: the short term time frame for assessing affordability versus the long-term timeframe for assessing value; variability in willingness to pay (and applicability of ICER threshold) based on degree of unmet medical need addressed; and the subjectivity of incorporating contextual considerations such as caregiver and societal impacts into a quantitative framework.	ICER	Feb 2019
Comments on the FDA's "Framework for a Real-World Evidence Program"	ARM's comments encourage further clarification of the use of real world evidence (RWE) in satisfying post-approval requirements for products with RMAT designation. ARM also believes that the RWE framework document should take incorporate special considerations to address issues specific to the use of RWE for RMAT products.	FDA	Feb 2019



RMAT Designation

Creation of the RMAT designation to bring safe & effective therapies to market quickly



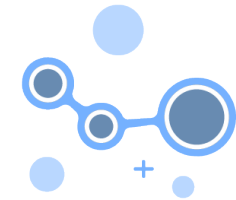
Gene Therapy

Inclusion of gene therapies in the RMAT designation



RAC Reform

NIH ended duplicative review of gene therapy trials



Gene Editing

Published Therapeutic Developers Statement of Principles