Meeting on the Mediterranean 2019

Day 2: Welcome Remarks

Matt Patterson
Chairman & CEO, Audentes Therapeutics
Chairman, Alliance for Regenerative Medicine

April 24, 2019

Alliance *for* Regenerative Medicine

Access to Meeting Content

-- All presentations, talks, & panels available through livestream: www.meetingonthemed.com/webcast

-- Recordings will be accessible shortly at www.meetingonthemed.com, and

-- ARM presentations, publications, webinars, and other materials: www.alliancerm.org

Follow ARM on Twitter!
@alliancerm

Alliance for Regenerative Medicine

Role as ARM Chairman

- Collaborate with CEO and Board on strategic vision for ARM and facilitate consensus among board members
- Assist ARM team in establishment of short and medium-term goals and initiatives
- Provide support and feedback to ARM CEO and staff in the implementation of plans to achieve objectives
- Assist in the creation of a unified voice for the sector

Day 1 Key Takeaways

- Robust clinical pipeline, with several products expected to come to market in the short term and many in late-stage clinical development
- Strong and sustained investor interest in recent years
- Overall positive regulatory environment in Europe and globally
    - Regulators are interested in working with sector stakeholders to promote patient access to safe and effective therapies
- Significant challenges related to commercialization and patient access exist, necessitating collaboration between industry, payors, regulators, and other stakeholders

Select Anticipated Near-Term Approvals Globally

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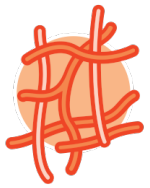

Enzyvant Therapeutics' **RVT-802** a tissue-based product for the treatment of complete DiGeorge Anomaly
Decision expected: 2019 (US)

AveXis / Novartis's **Zolgensma** gene therapy for the treatment of spinal muscular atrophy type 1
Decision expected: May 2019 (US), mid-2019 (EU), mid-2019 (Japan)

Kiadis Pharma's **ATIR101** gene modified cell therapy for the treatment of leukemia
Decision expected: 1H 2019 (EU)

bluebird bio's **Zynteglo** gene therapy for the treatment of beta thalassemia
Decision expected: mid-2019 (EU)
Expects to file (US) in 2019

PTC Therapeutics' **GT-AADC** gene therapy for the treatment of aromatic L-amino acid decarboxylase (AADC) deficiency
Expects to file: late 2019 (US)

Mesoblast's **Remestemcel-L** stem cell therapy for the treatment of acute graft versus host disease
Decision expected: 2020 (U.S.)

Mesoblast's / JCR Pharma's **TEMCELL** stem cell therapy for the treatment of epidermolysis bullosa
Decision expected: 2020 (Japan)

Orchard' Therapeutics's **OTL-101**, a gene therapy for the treatment of ADA Deficiency / ADA-SCID
Expects to file: 2020 (US)

Orchard Therapeutics's **OTL-200**, a gene therapy for the treatment of meta-chromatic leukodystrophy
Expects to file: 2020 (US & EU)

Market Access Landscape in 8 Countries

As of January 2019

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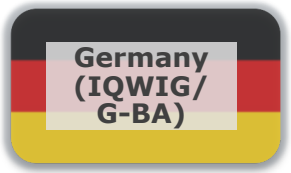
Kymriah ★

Yescarta ★



Alofisel ★ ✓

Imlygic ✓

Kymriah ✓

Luxturna ✓

Strimvelis ✓

Yescarta ✓

Zalmoxis ★ ✓

Holoclar ★

Imlygic ★ ✓

Kymriah ★ ✓

Strimvelis ★ ✓

Yescarta ★ ✓

Holoclar ★

Imlygic
Authorized, hospital only

Strimvelis ★ ✓

Zalmoxis ★ ✓

Imlygic ★ ✓

Imlygic ★ ✓

Kymriah ★ ✓

Luxturna ★ ✓

Yescarta ★ ✓

Kymriah
CADTH Assessment: Would be cost effective if price lowered

Kymriah ✓

★ *Positive HTA/Payor Opinion* ✓ *Reimbursed*

Ensuring Patient Access to ATMPs

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1. Developing and implementing innovative pricing & reimbursement models

- Because these therapies have the potential to provide durable or even curative effects, they may provide immense long-term value
- Existing models for reimbursement are not adequately equipped to accommodate ATMPs
- Traditional HTA models may not recognize the value of one-time curative therapies
- Alternate financing models – such as payment-over-time and pay-for-performance models – help payers absorb higher cost therapies and offset risk

2. Improving healthcare infrastructure

- Ensuring medical centers are well trained and prepared to administer ATMPs and follow patients post-treatment
- Improving the infrastructure for long-term patient follow up and measurement of required outcomes

3. Addressing issues with manufacturing and supply-chain processes

- Increased demands on manufacturing and supply chain systems as more ATMPs are approved, especially in indications with large patient populations
- Storage and handling requirements, as well as value of ATMPs, likely will lead to limited distribution and pharmacy models
- Need for international regulatory convergence

Day 2 Agenda

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Panel topics:

- Recent Developments and Predictions for the Future of Advanced Therapies
- Gene Editing: Partnering for Success
- European Clinical Trials in a Global Context
- Fireside Chat: Regulating ATMPs in a Post-Brexit World

Coming Up Next:

Recent Developments and Predictions for the Future of Advanced Therapies

Chair:
Morrie Ruffin, Co-Founder and Senior Advisor, ARM

Speakers:
Amy DuRoss, Co-Founder and CEO, Vineti
Sven Kili, M.D., Principal, Sven Kili Consulting
John Tchelingerian, Ph.D., President and CEO, Promethera Biosciences
Christopher Vann, Chief Operating Officer, Autolus Therapeutics
Rogerio Vivaldi, M.D., President and CEO, Sigilon Therapeutics