



# Welcome

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## Cell & Gene Meeting on the Mesa

*Janet Lambert, CEO ARM*

# Thank You to the ARM Events Team!



**Laura Parsons**

*Senior Director, Marketing And Events*



**Bethany Kraynack**

*Vice President, Operations*



**Chelsey Hathaway**

*Director, Patient Engagement And Events*



**Jeanette Lazusky**

*Coordinator, Events*

# ARM's 10th Anniversary



# Patient Impact of Recently Approved Products

Therapy Name	Product Developer	Response
<b>Kymriah</b>	Novartis	<ul style="list-style-type: none"> <li><b>40%</b> of patients with R/R DLBCL treated experienced a complete response</li> <li><b>60%</b> of patients with R/R B-Cell ALL treated experienced a complete response</li> </ul>
<b>Yescarta</b>	Kite Pharma, a Gilead company	<ul style="list-style-type: none"> <li><b>58%</b> of patients with R/R B-Cell NHL treated experienced a complete response</li> </ul>
<b>LUXTURN A</b>	Spark Therapeutics	<ul style="list-style-type: none"> <li><b>55%</b> of patients treated showed an improvement of 2+ light levels darker after treatment</li> </ul>
<b>Zolgensma</b>	AveXis / Novartis	<ul style="list-style-type: none"> <li><b>93%</b> of patients SMA Type 1 treated were alive without permanent ventilation at 24 months post-treatment</li> </ul>
<b>Zynteglo</b>	bluebird bio	<ul style="list-style-type: none"> <li><b>75%</b> of patients with TDT without <math>\beta 0/\beta 0</math> genotype treated achieved transfusion independence</li> </ul>







## Gene Therapy

### **Zolgensma** (AveXis / Novartis)

- Spinal muscular atrophy type 1
- Decision expected: mid 2019 (EU & Japan)

### **GT-AADC** (PTC Therapeutics)

- AADC deficiency
- Expects to file: late 2019 (US)

### **Zynteglo** (bluebird bio)

- Beta thalassemia
- Expects to file: 2019 (US)

### **Valrox** (BioMarin)

- Hemophilia A
- Expects to file: Q4 2019 (US & EU)

### **GS010** (GenSight Biologics)

- Leber hereditary optic neuropathy
- Expects to file: H2 2020 (US & EU)

### **AT132** (Audentes Therapeutics)

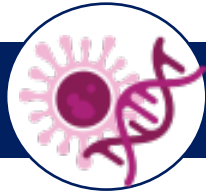
- X-linked myotubular myopathy
- Expects to file: H2 2020 (US)

### **OTL-101** (Orchard Therapeutics)

- ADA-SCID
- Expects to file: 2020 (US)

### **OTL-200** (Orchard Therapeutics)

- Metachromatic leukodystrophy
- Expects to file: 2020 (US & EU)



## Cell-Based Immuno-Oncology

### **Rivo-cel** (Bellicum Pharmaceuticals)

- HSCT to treat blood cancers
- Expects to file: EOY 2019 (EU)

### **tab-cel** (Atara Biotherapies)

- EBV-PTLD
- Expects to file: 2H 2019 (US)

### **liso-cel** (Celgene)

- Diffuse large B-cell lymphoma (DLBCL)
- Expects to file: Q4 2019 (US)

### **ide-cel** (bluebird bio / Celgene)

- Multiple myeloma
- Expects to file: 1H 2020 (US)

### **ATIR101** (Kiadis Pharma)

- HSCT to treat blood cancers
- Decision expected: 1H 2020 (EU)

### **P-BCMA-101** (Poseida Therapeutics)

- Multiple myeloma
- Expects to file: 2020 (US)

### **Lifileucel** (Iovance)

- Advanced metastatic melanoma
- Expects to file: 2020 (US)

### **LN-145** (Iovance)

- Advanced metastatic cervical cancer
- Expects to file: 2H 2020 (US)

# Select Anticipated Near-Term Approvals (Global)



## Cell Therapy

### **SB623** (SanBio)

- Traumatic brain injury
- Expects to file: January 2020 (Japan)

### **Remestemcel-L** (Mesoblast)

- Acute graft versus host disease
- Decision expected: 2020 (US)

### **TEMCELL** (Mesoblast / JCR Pharma)

- Epidermolysis bullosa
- Decision expected: 2020 (Japan)



## Tissue-Based

### **RVT-802** (Enzyvant Therapeutics)

- Complete DiGeorge anomaly
- Decision expected: 2019 (US)

### **Humacyl** (Humacyte)

- End stage renal disease
- Expects to file: 2020 (US)

### **Stratagraft** (Mallinckrodt)

- Deep partial thickness burns
- Expects to file: 2020 (US)