



Resilience in the Face of COVID-19

Janet Lambert, CEO

AusBiotech + Invest 2020
October 21, 2020





About ARM

Promote Clear Regulation

Enable Innovative Reimbursement

Address Manufacturing Barriers

Educate Stakeholders





Recently Approved Products

Kymriah: 40% to 82% complete response rates

Luxturna: improved vision in 93% of patients

Tecartus: 62% complete response rate

Yescarta: 58% complete response rate

Zolgensma: 93% of patients alive & ventilator free

Zynteglo: 75% of patients transfusion-free





Sector Landscape & Trends



1,001
TOTAL GENE, CELL & TISSUE-
BASED THERAPEUTIC
DEVELOPERS WORLDWIDE

543

238

184

13

1

23





\$10.7B raised in the first half of 2020

- Surpassing all of 2019
- On track to break financing records



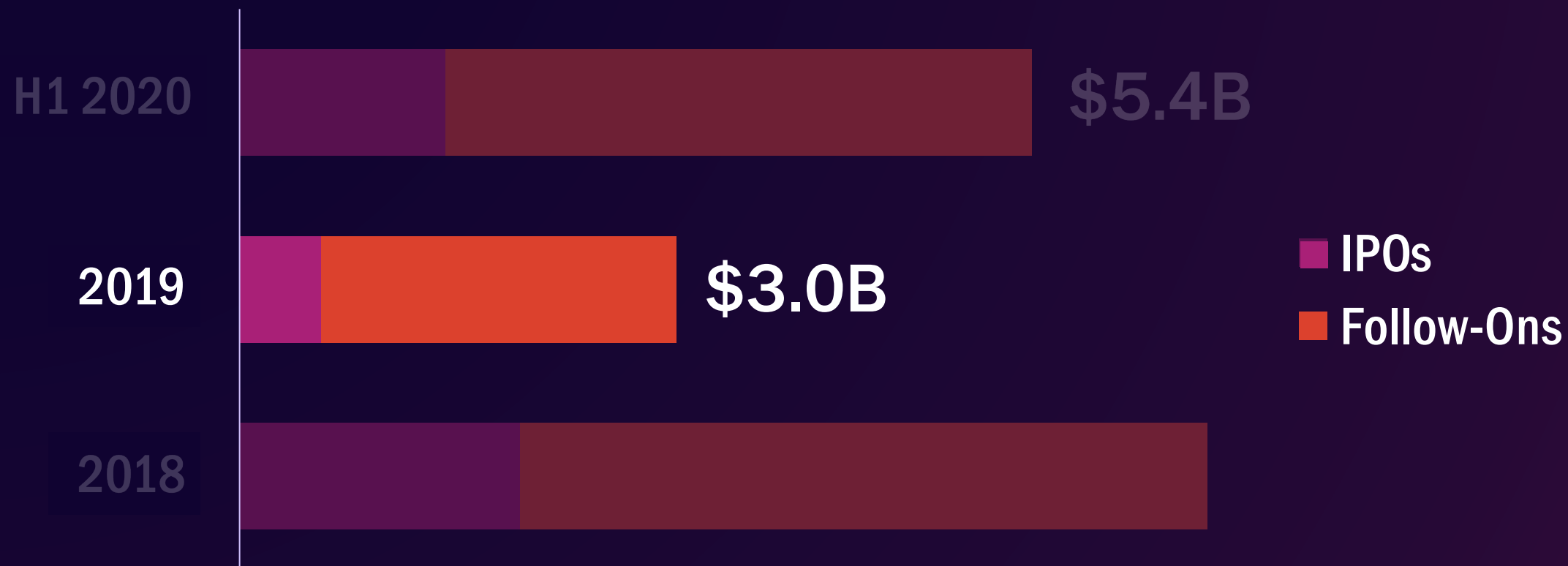
Public financing is extremely strong.



Five companies went public in H1 2020



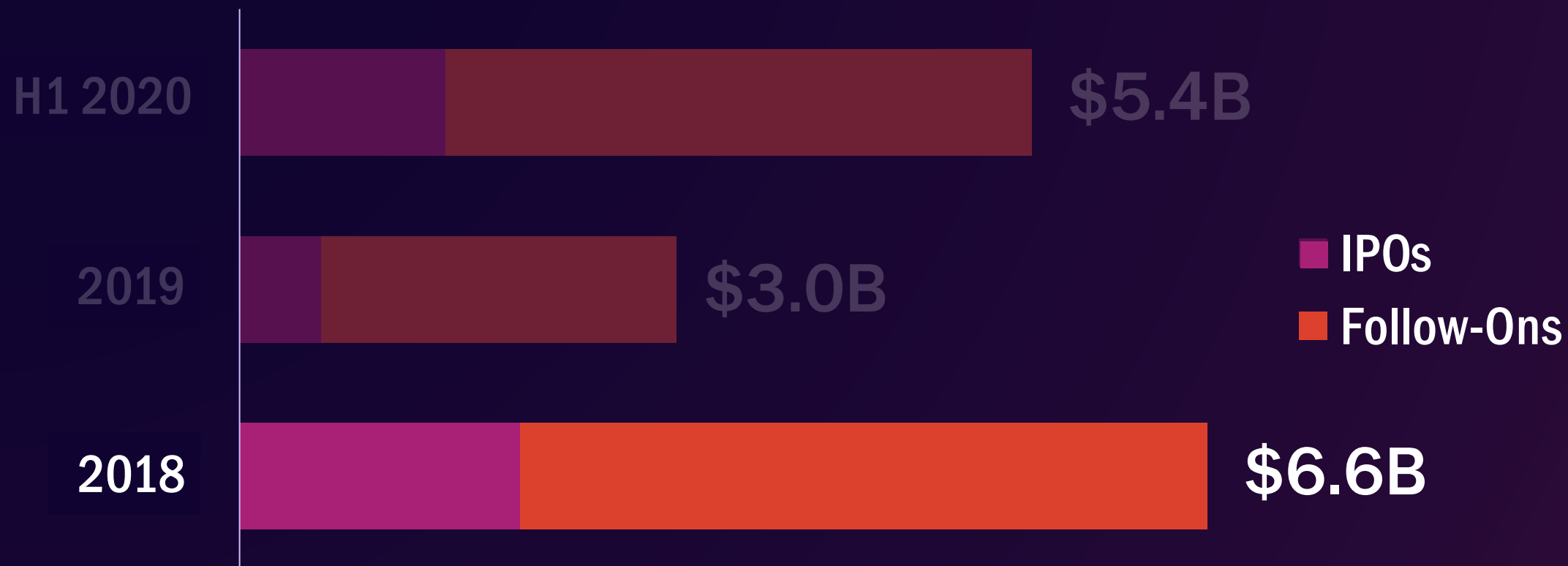
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Venture capital is also plentiful.



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\$100M+ Financings Flourish

PRIVATE FINANCINGS

- Sana Bio – \$700M (Jun 23)
- Orca Bio – \$192M (Jun 17)
- Elevate Bio – \$170M (March 30)
- Legend Bio – \$150M (Apr 1)
- Freeline Tx – \$120M (Jun 30)
- Poseida Tx – \$110M (Jun 25)
- Generation Bio – \$110M (Jan 10)
- Akouos – \$105M (March 3)
- JW Tx – \$100M (June 9)

FOLLOW-ON FINANCINGS

- Iovance – \$604M (Jun 2)
- bluebird – \$575M (May 18)
- Allogene – \$550M (Jun 1)
- Adaptimmune – \$259M (Jun 4)
- Editas – \$216M (June 23)
- Atara Bio – \$202M (Jun 24)
- Fate Tx – \$201M (Jun 11)
- Adverum Bio – \$150M (Feb 14)
- Krystal Bio – \$125M (May 18)
- IVERIC bio – \$125M (Jun 17)
- Intellia Tx – \$115M (Jun 5)
- Replimune – \$115M (Jun 8)
- AVROBIO – \$100M (Feb 18)

INITIAL PUBLIC OFFERINGS

- Legend Bio – \$487M (Jun 9)
- Passage Bio – \$284M (Feb 3)
- Akouos – \$244M (Jun 25)
- Generation Bio – \$230M (Jun 12)
- Beam Tx – \$207M (Feb 11)

CORPORATE PARTNERSHIP (UPFRONT PAYMENTS ONLY)

- uniQure & CSL Behring – \$450M (Jun 24)
- Biogen & Sangamo – \$350M (Feb 27)
- bluebird & Bristol-Myers Squibb – \$200M (May 11)
- UltraGenyx & Daiichi Sankyo – \$125M (Mar 31)
- Fate Tx & Janssen – \$100M (Apr 2)
- Regeneron & Intellia – \$100M (Jun 1)



**You treat a patient once
and they're done.**



Gbola Amusa, Chardan

ARM's Biotech Fundraising Amidst COVID-19 webinar



Reaching new milestones in gene editing

- 9-month data for gene-edited therapy for sickle cell disease
- First *in vivo* CRISPR patient treated
- Positive initial data from gene-edited allogeneic cell therapies for cancer





COVID-19 Vaccines

Corporate Partnerships

In-House Manufacturing

New CMOs Launch

Capacity Building

**Developers & manufacturers
are working to increase
capacity.**





COVID-19 Vaccines

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- Merck KGaA amps up in gene therapy with \$110M viral vector manufacturing boost
- Catalent to invest \$130M in gene therapy plant near Baltimore airport
- Catalent chief triggers a \$315M-plus acquisition of cell therapy CMO MastherCell
- Emergent plots \$75M expansion to produce viral vectors for expanding gene therapy business
- Bio-Techne: \$50m reagent plant will support cell & gene therapies
- RoslinCT Announces the Construction of a New Cell & Gene Therapy Manufacturing Facility



Capacity Building



COVID-19 Vaccines

Corporate Partnerships

In-House Manufacturing

- Real estate group MLP Ventures creating \$1.1B gene therapy CDMO at former GSK site
- Nationwide Children's, a gene therapy leader, launches manufacturing spinout
- Bio-Techne, Fresenius Kabi, Wilson Wolf Form Joint Venture for Cell and Gene Therapy



New CMOs Launch

Capacity Building



COVID-19 Vaccines

Corporate Partnerships

- Audentes investing \$109M in gene therapy manufacturing facility with 200 jobs
- Beam Therapeutics to build \$83M manufacturing facility, create hundreds of jobs
- Forge Biologics Launches With \$40M Series A Financing to Manufacture and Develop Gene Therapies
- Krystal Biotech Breaks Ground On Second Commercial Gene Therapy Manufacturing Facility
- CRISPR Therapeutics Announces the Build-Out of New Cell Therapy Manufacturing Facility
- Marker Tx Announces New Manufacturing Facility to Support Clinical Development of T Cell Therapy Candidates



In-House Manufacturing

New CMOs Launch

Capacity Building



COVID-19 Vaccines

- Vivet Therapeutics and Pfizer enter into manufacturing agreement for gene therapy for Wilson disease
- Ultragenyx enters \$200M upfront agreement with Daiichi Sankyo for gene therapy manufacturing technology`
- Oxford Biomedica signs agreement with Beam Tx for LentiVector platform for next-generation CAR-T therapeutics
- Thermo Fisher Scientific and Lyell Immunopharma form cell therapy manufacturing partnership
- Catalent enters into strategic partnership with Editas Medicine to support gene-editing medicine pipeline



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- **CGT Catapult nabs \$127M in U.K. funding to gear up for large-scale COVID-19 vaccine production**
- **Oxford Biomedica signs five-year collaboration agreement with the Vaccines Manufacturing and Innovation Center**
- **Univercells' raises \$50M for spin-off CDMO Exothera to help scale COVID-19 vaccine manufacturing**



COVID-19 Vaccines

Corporate Partnerships

In-House Manufacturing

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Capacity Building

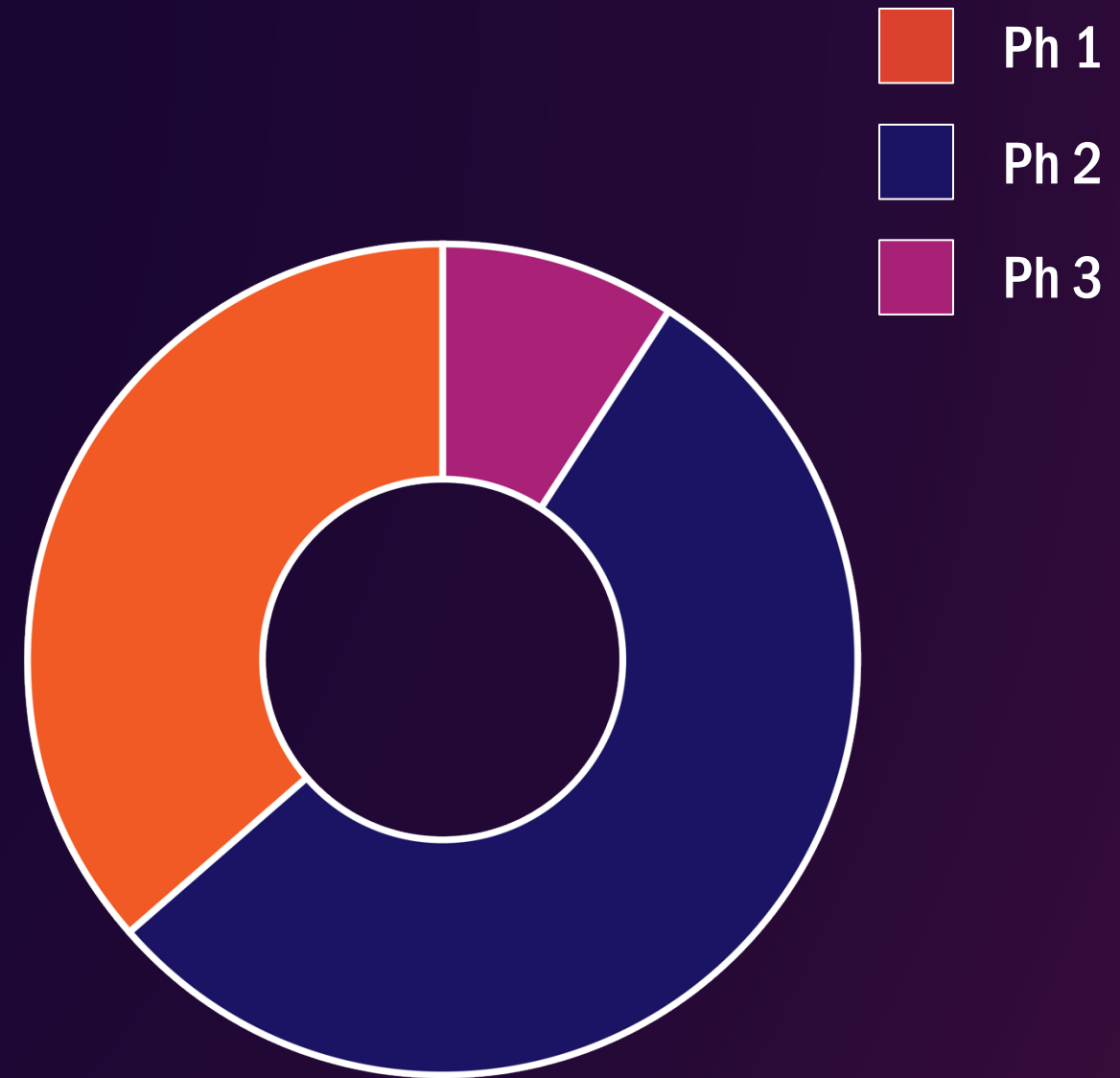


Manufacturing remains one of the key regulatory hurdles to approval for many developers.



The pipeline continues to grow & advance.

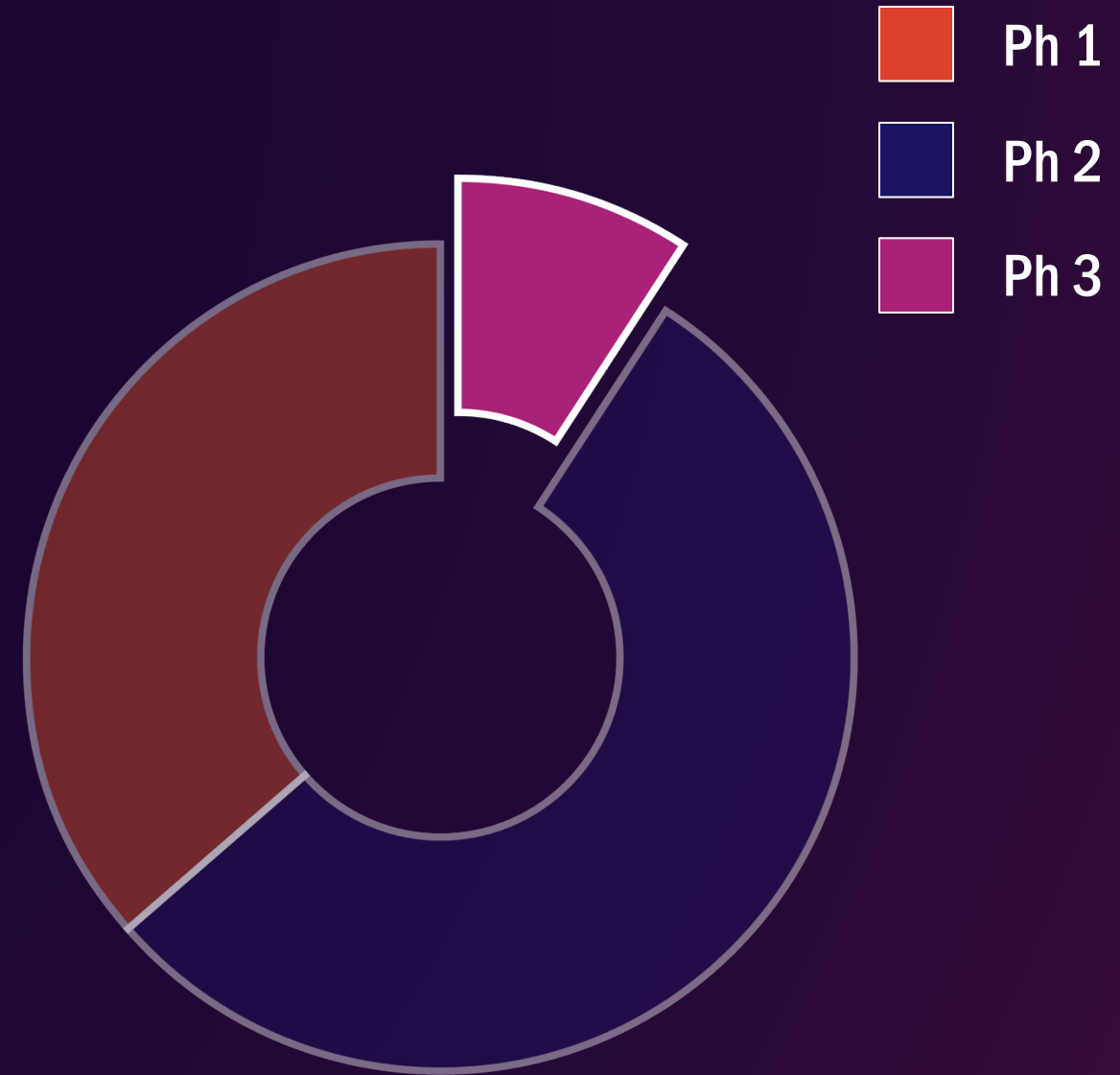
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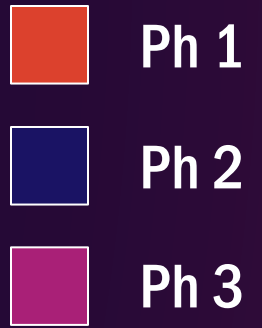
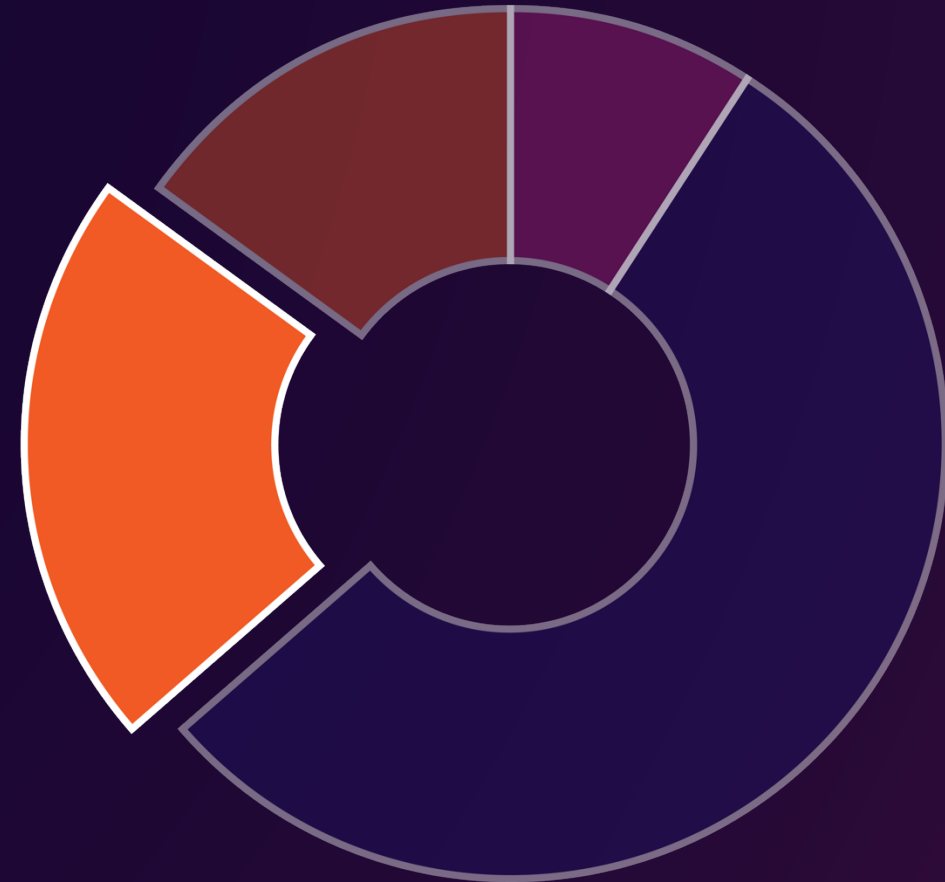
- 97 clinical trials in Phase 3



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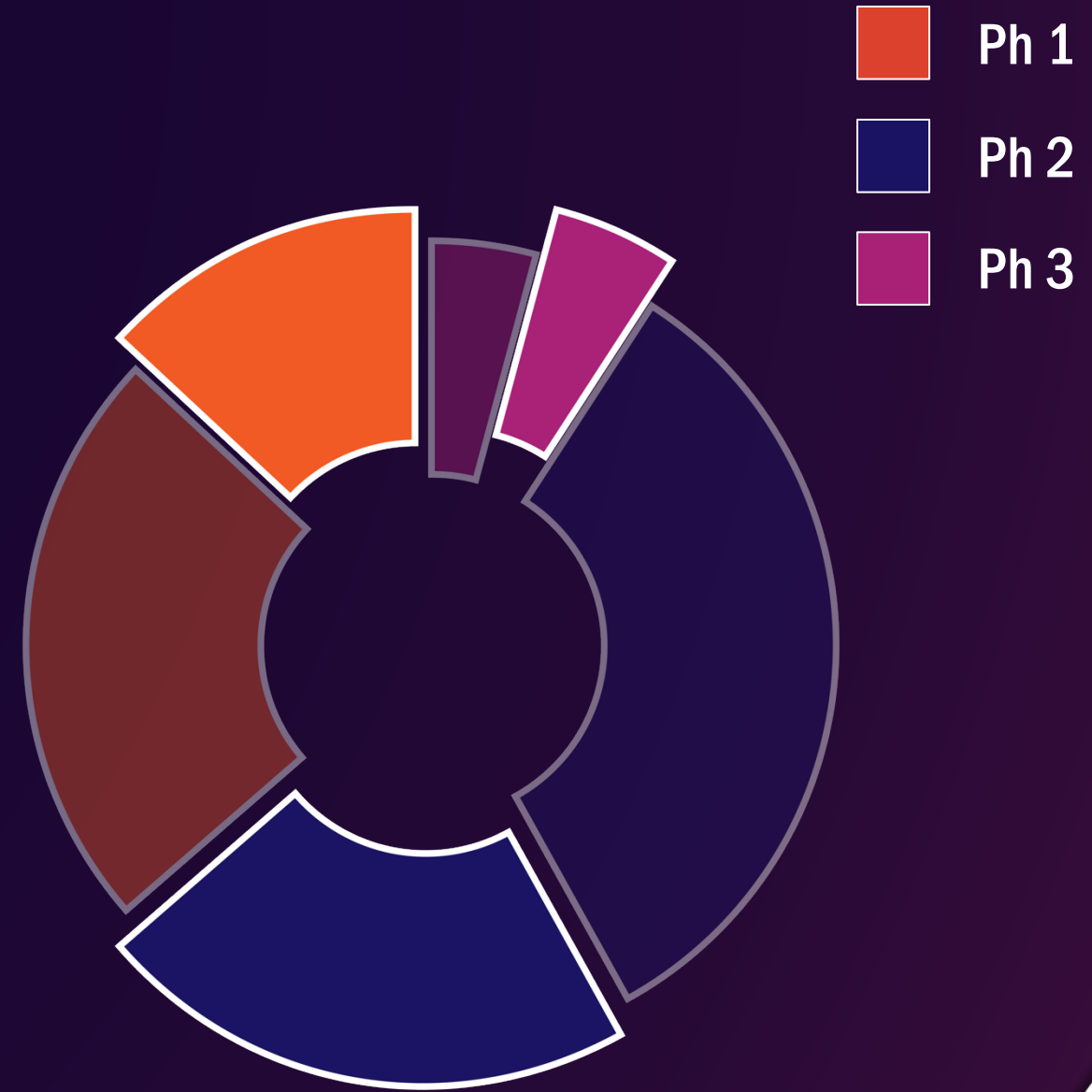
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- Cell-based immuno-oncology makes up growing proportion of the pipeline



The pipeline continues to grow & advance.

1,079 total ongoing clinical trials, including:

- 97 clinical trials in Phase 3
- Cell-based immuno-oncology makes up growing proportion of the pipeline
- Growing number of clinical trials for more prevalent disorders



International regulators remain committed to innovative therapies.

- EMA & FDA have reiterated expectation of 20 approvals/applications annually by 2025
- Decisions on several new marketing applications expected in coming months



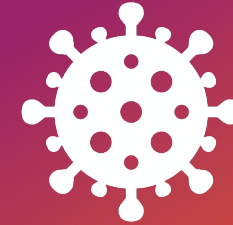
Potential upcoming approvals include:



Libmeldy (Orchard Tx)
Early-onset metachromatic leukodystrophy



GT-AADC (PTC Bio)
AADC deficiency



Ide-cel (bluebird, BMS)
Relapsed or refractory multiple myeloma



Eli-cel (bluebird bio)
Cerebral adrenoleukodystrophy



Lumevoq (GenSight Bio)
Leber hereditary optic neuropathy



International regulators remain committed to innovative therapies.

- EMA & FDA have reiterated expectation of 20 approvals/applications annually by 2025
- Decisions on several new marketing applications expected in coming months
- However, COVID-19 could cause delays





Promoting Market Access

Overcoming structural barriers to innovative payment models





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Overcoming structural barriers to innovative payment models

Creating frameworks for the evaluation of durable therapies





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Overcoming structural barriers to innovative payment models

Creating frameworks for the evaluation of durable therapies

Implementing RWE and other infrastructure for long-term follow up





Thank you.

These slides will be available on www.alliancerm.org

