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Manufacturing Challenges Facing Cell & Gene Therapy

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Increased Need for Large-Scale Manufacturing

Mike Lehmicke,
Director, Science & Industry Affairs, ARM

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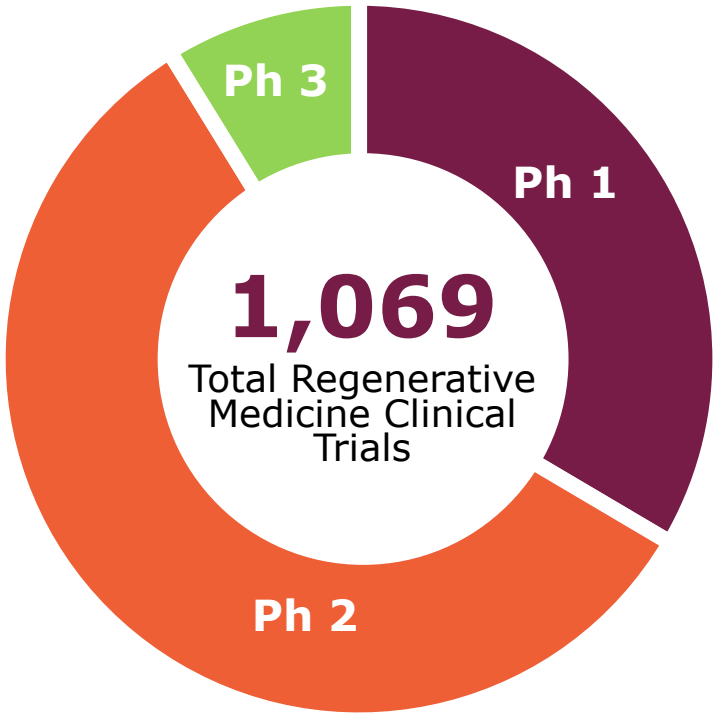
"We anticipate that by 2020 we will be receiving **more than 200 INDs per year** […] And by 2025, we predict that the FDA will be **approving 10 to 20 cell and gene therapy products a year** based on an assessment of the current pipeline and the clinical success rates of these products."

– Statement from Former FDA Commissioner Scott Gottlieb and CBER Director Peter Marks on new policies to advance development of safe and effective cell and gene therapies

"I had the opportunity to spend some time with my colleagues at the FDA and we've seen a steady growth in clinical trials. We **both expect to grow to 10 to 20 product approvals or submissions each year** within the next five years."

– Guido Rasi, Director General of the EMA, during his remarks at ARM's 2019 Meeting on the Mediterranean

Increasing Numbers of Clinical Trials



Phase 1: 358
across all tech types and indications

Gene Therapy: 117
Gene-Modified Cell Therapy: 187
Cell Therapy: 49
Tissue Engineering: 5

Phase 2: 617
across all tech types and indications

Gene Therapy: 219
Gene-Modified Cell Therapy: 207
Cell Therapy: 168
Tissue Engineering: 23

Phase 3: 94
across all tech types and indications

Gene Therapy: 30
Gene-Modified Cell Therapy: 16
Cell Therapy: 32
Tissue Engineering: 16

Source data provided by: informa

Increasing Focus on Indications with Large Patient Populations

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- Consider clinical and preclinical development in the cardiovascular, central nervous system, and diabetes space

- First gene therapy for critical limb ischemia approved in Japan this year

- Clinical trials in cardiovascular and CNS/neurological indications:
    - 23 in diabetes and related complications
    - 11 in Parkinson's disease
    - 10 in critical limb ischemia
    - 8 in stroke
    - 7 in peripheral artery disease
    - 6 in heart failure
    - 5 in heart attack
    - 5 in Alzheimer's disease

Expedited Approval Programs Shorten Development Timelines

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34 RM/AT therapies have RMAT Designation

18 RM/AT therapies have Breakthrough Designation

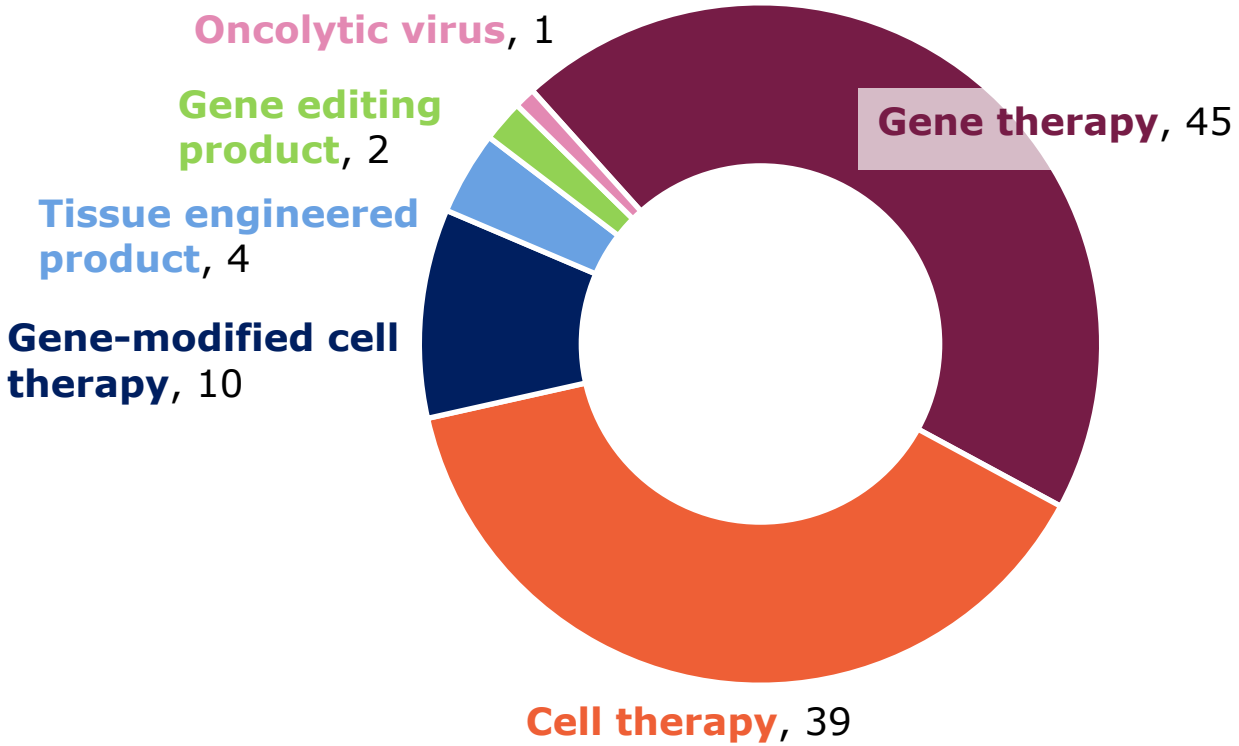
34 RM/AT therapies have Fast Track Designation

4 RM/AT therapies have SAKIGAKE Designation

19 RM/AT therapies have PRIME Designation

Products with Expedited Approval

By technology type



Expedited Approval Programs Shorten Development Timelines

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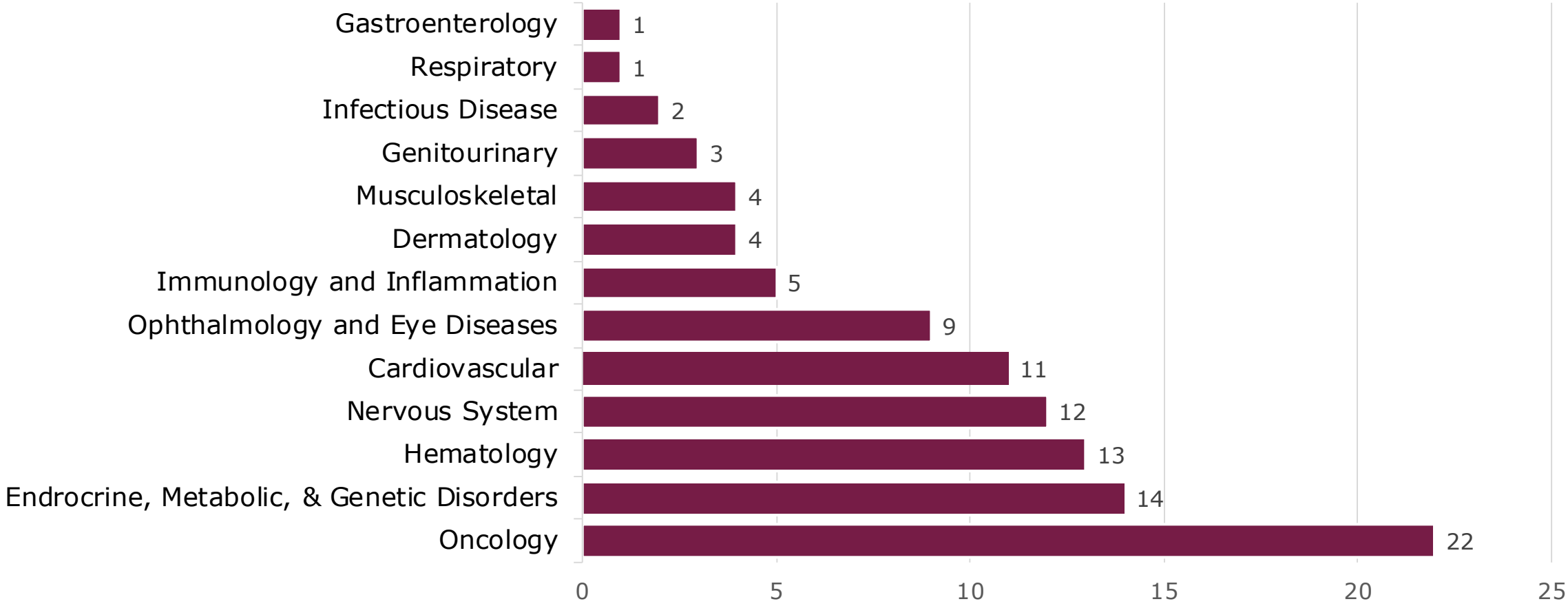
Products with Expedited Approval

By indication area



Panel Discussion

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Topic 1: Transitioning Manufacturing from Small- to Large-Scale

In transitioning from smaller, pilot-scale production to larger, commercial-scale manufacturing, what are the biggest challenges?

Best strategies and lessons learned?

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Topic 2: In-House Vs. Outsourced Manufacturing

Advantages / disadvantages to building out internal manufacturing capabilities or outsourcing manufacturing.

At what stage of a company's life should it be thinking about building or outsourcing?

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Topic 3: Dealmaking

What is behind the numerous consolidating deals in the cell & gene therapy manufacturing market YTD in 2019?

Can we expect this activity to continue?

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Topic 4: New and Innovative Ways to Manufacture Cell/Gene Therapy for the Future

New and innovative ways companies are investigating to manufacture cell and gene therapies

Examples of innovative technologies to enable a robust and reproducible manufacturing process include a closed and/or automatic process

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Topic 5: Standards for New Cell and Gene Therapy Products

FDA says manufacturers should rely more on standards to ease development and approval process – however, development of standards has been a slow process.

What are the next steps in establishing standards?

How will the gap in deciding on certain standards be filled in the meantime?

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Topic 6: Logistical Challenges with Cell Therapy Manufacturing

What are the current key challenges and how can developers overcome them?

Topic 7: Challenges with Vector Production

What are the biggest challenges with viral vector production?

Discuss next-generational materials and non-viral delivery methods.

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Thank you!

Questions?