TuftsMedicine
Tufts Medical Center

Durable Cell and Gene Therapy Comparative Success Rates

Oct 11, 2023

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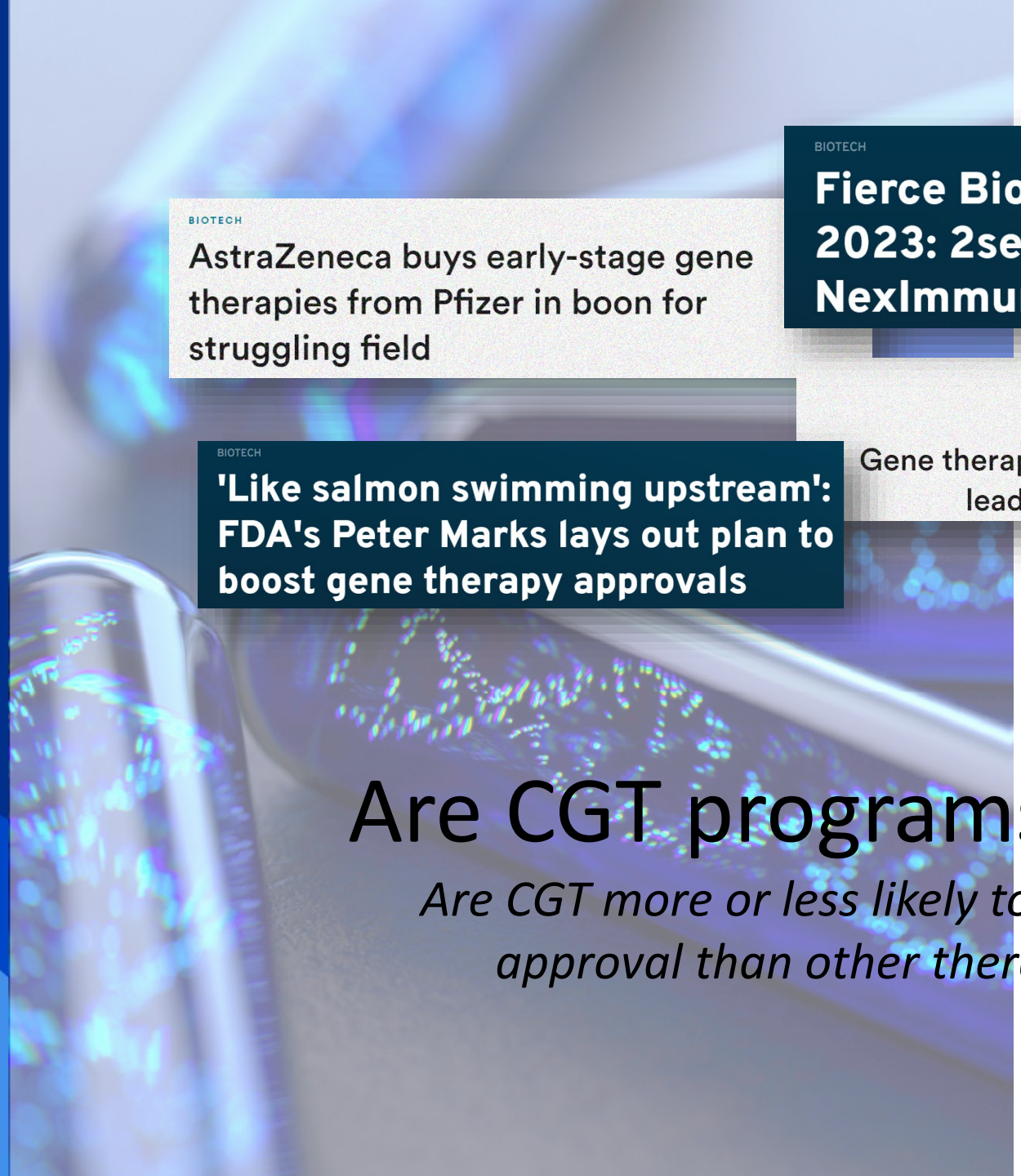

Are CGT programs a better bet?

Are CGT more or less likely to move from Phase 1 to approval than other therapeutic modalities?

NEWDIGS – Helping the System Catch Up With the Science

Drive more value faster to patients, in ways that work for all stakeholders

- Safe haven **"think & do" tank**
- Track record of **real-world impact**
- Interactive methods/tools for **multi-stakeholder design**
- Bold, transformational system innovations **for 14 years**
- Founded at MIT, joined Tufts Medical Center in July 2022

NEWDIGS "Adaptive Licensing" Project fueled timely action & impact in Europe from regulatory science innovation…..

STATE OF THE ART
nature publishing group
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See COMMENTARY page 378

Adaptive Licensing: Taking the Next Step in the Evolution of Drug Approval

H-G Eichler[1,2], K Oye[2,3,4], LG Baird[2], E Abadie[5], J Brown[6], CL Drum[2], J Ferguson[7], S Garner[8,9], P Honig[10], M Hukkelhoven[11], JCW Lim[12], R Lim[13], MM Lumpkin[14], G Neil[15], B O'Rourke[16], E Pezalla[17], D Shoda[18], V Seyfert-Margolis[14], EV Sigal[19], J Sobotka[20], D Tan[12], TF Unger[18] and G Hirsch[2]

… and Illuminated a Broad Set of Principles for Accelerating Sustainable Patient-Centered Innovation

LEAPS

Learning Ecosystems Accelerator for Patient-centered, Sustainable innovation

FoCUS

Financing and Reimbursement of Cures in the US

Clinical Pharmacology & Therapeutics (2012); **91** 3, 426–437. doi:10.1038/clpt.2011.345

FoCUS Project: *Dedicated to making innovative cures accessible and sustainable*

Durable, potentially curative therapies for genetic disorders and cancer have arrived.

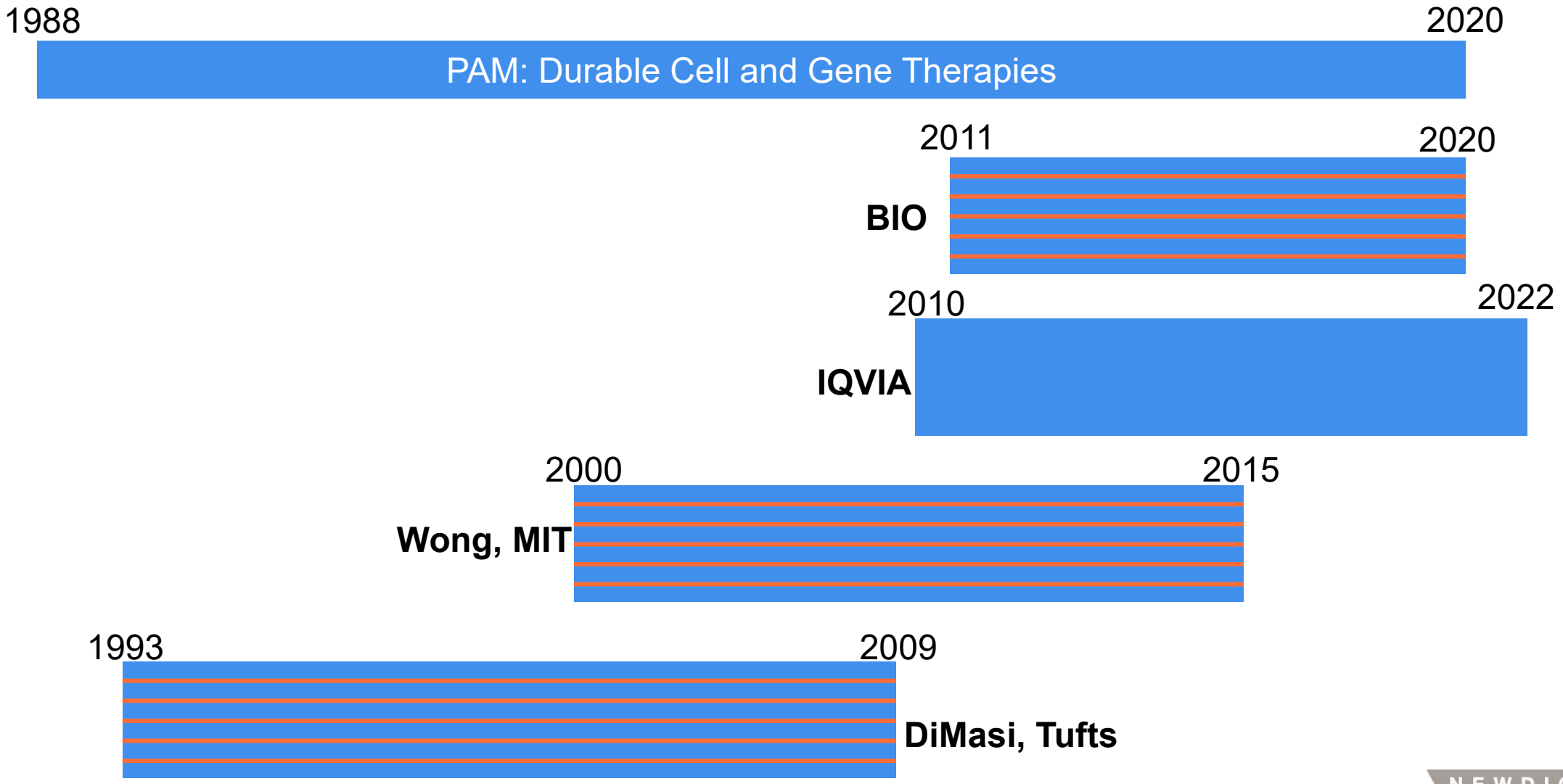
Since 2016, >120 organizations & 420 individuals engaged to envision and evolve payment models, regulations, and business operations.

The FoCUS Consortium designs and shares precision financing solutions to ensure patient access and system sustainability.

FoCUS does not address therapy value or price.

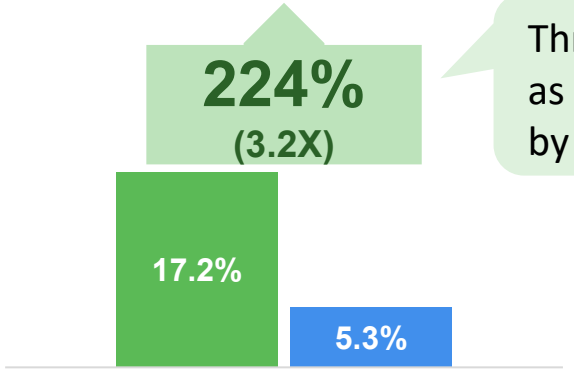
We Compared FoCUS Pipeline Analysis Model to BIO and IQVIA Success Rates, and some Academics

Pipeline coverage varied among all estimates. We emphasize more recent BIO and IQVIA data.



CAR-T/TCR therapies for blood cancers are 3 times as likely to be approved as the average oncology drug

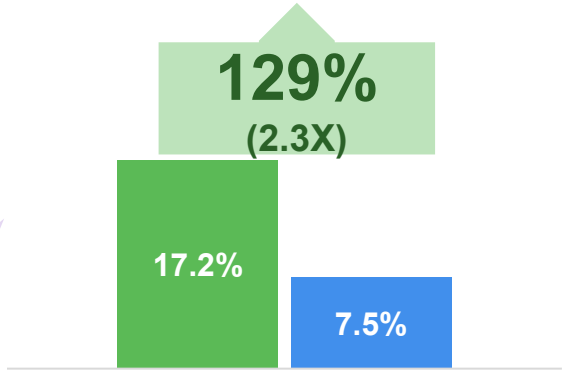
How do they perform compared to the average oncology drug?



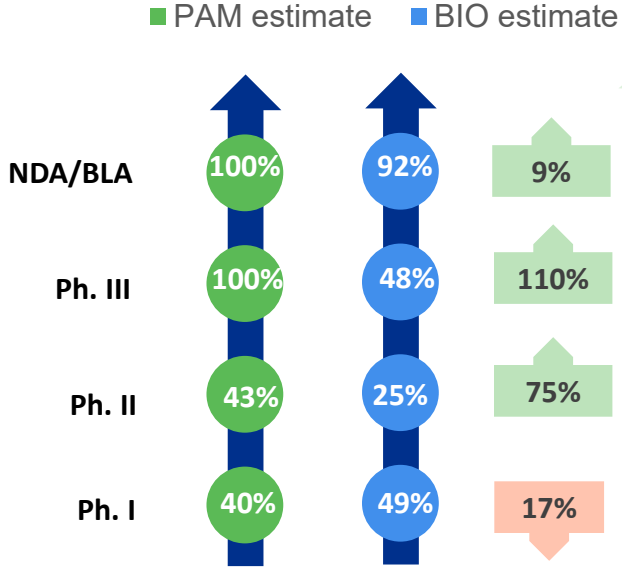
Three times as likely to be approved as the average oncology drug studied by BIO

More than double the likelihood of approval compared to only hematological oncology drugs studied by BIO

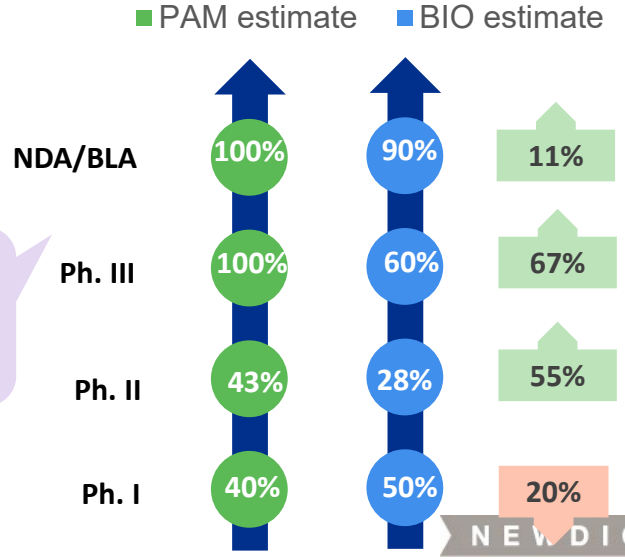
How do they perform compared to the average hematological oncology drug?



After entering Phase II, outperforms the average oncology drug at every phase



Better by-phase success rates compared to other blood cancer drugs, in Phase II onwards



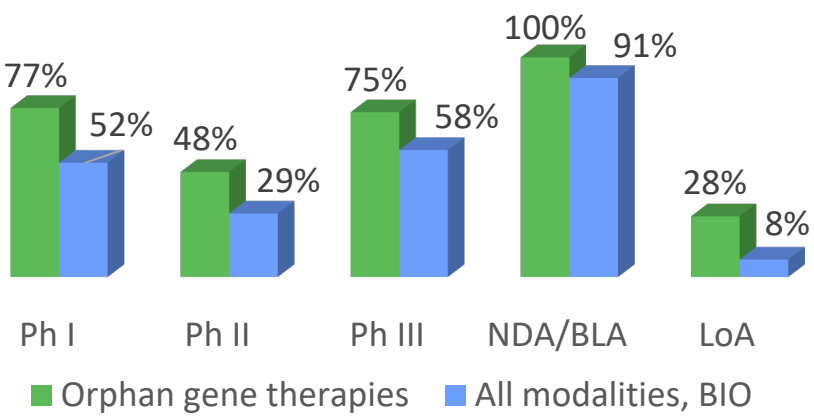
Orphan gene therapies are 2 – 3.5X as likely to be approved as the average drug in clinical trials, outperforming in every phase

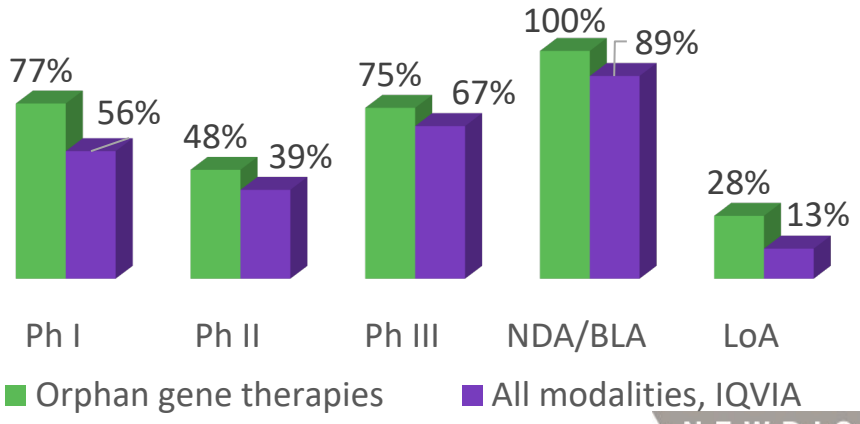
Orphan gene therapies Phase I LoA is 3.5x that of the BIO estimate for all medicines

Orphan gene therapies Phase I LoA is >2X that of the IQVIA estimate for all medicines

Ph. I likelihood of approval	249% (3.5X) higher	109% (2.1X) higher
NDA/BLA	10% higher	12% higher
Phase III	30% higher	12% higher
Phase II	65% higher	23% higher
Phase I	48% higher	37% higher

Phase-by-phase success rates





LoA: Likelihood of Approval

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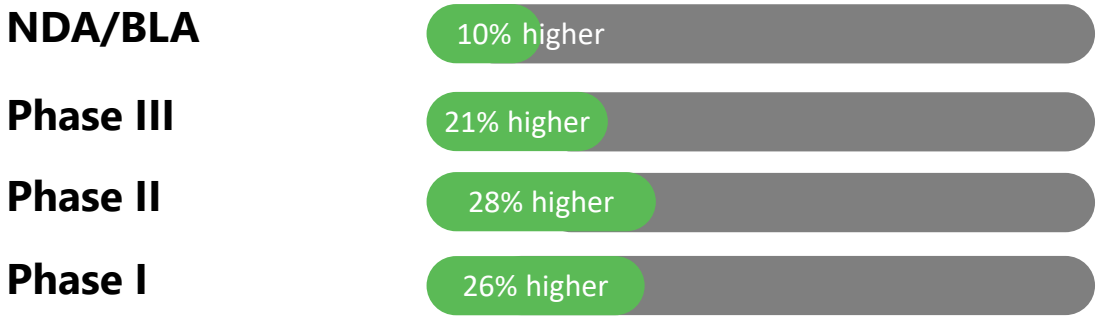
Orphan gene therapies are more than twice as likely to be approved as the average drug *in similar therapeutic areas**, outperforming in every phase

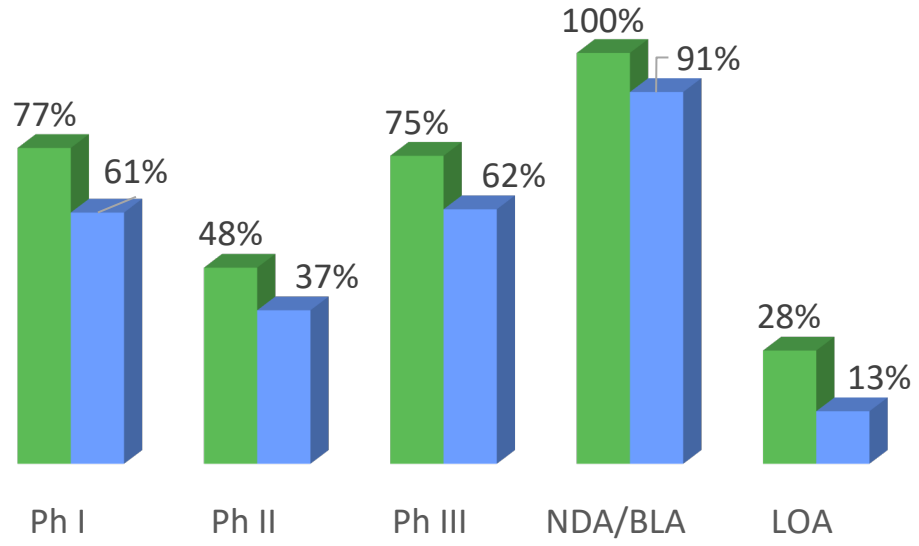
Overall likelihood of approval

115% higher (2.1X)

Clinical Phase Success Rates Percent Differences in Similar Therapeutic Areas



Clinical Phase Success Rates In Similar Therapeutic Areas

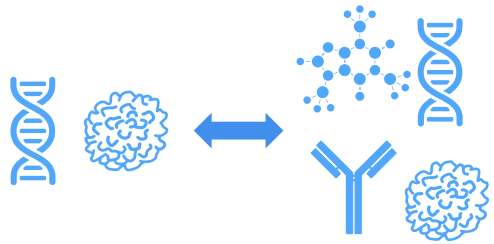


Orphan gene therapies (PAM estimates) compared to all modalities in similar therapeutic areas* (BIO estimates)

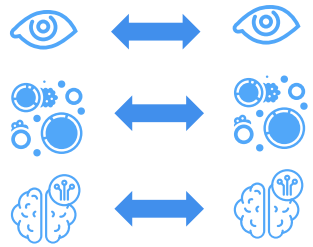
**Therapeutic areas for comparison: hematology, autoimmune, metabolic, neurology, ophthalmology*

Durable Cell and Gene Therapies ARE a Good Clinical Bet

- Durable CGT programs for rare orphan conditions and CAR-Ts for hematological cancers have higher success once they enter the clinic.
  This is consistent for comparisons to:
  - All therapeutic programs Likelihood of Approval



  - Programs in the same therapeutic areas



  - Every Development Phase (except Phase I for CAR-Ts)

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For Further Information Email Us At: TuftsmcNEWDIGS@tuftsmedicine.org

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