

Gene therapy innovation from platform to process

Our modular, next-generation AAV manufacturing platform is continuously evolving to meet developer needs in a rapidly evolving regulatory environment



PROCESSES DEVELOPED TO BALANCE Product quality, potency & yield

Innovation pipeline workflow

Discovery



Analytics



Selection framework



Asset / development candidate



Discovery Engine

Overexpression



Small molecules



CRISPR KO



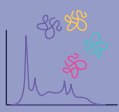
siRNA / miRNA



Omics



NGS

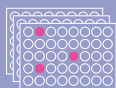


LC-MS

Candidate Selection



Validation



Hit ID



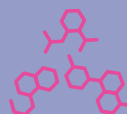
Analytics

Further iteration using
rational design

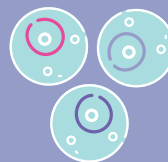


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ACTTAGTGACAGCTGT
ACTTGACTGACTAGCT
TGACTAGTGACTAGCT
TAGCTTAGTGACTAGC

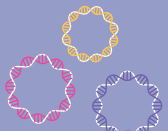
Areas of Innovation



Small molecule enhancers

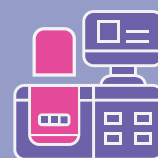


Enhanced production cells

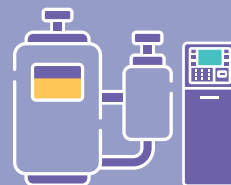


Improved plasmids

New Platform



Process development
& scale up



Client ready
process



 **ascend**



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ADVANCED THERAPY MANUFACTURING SPECIALISTS

200+

Bioreactor runs

3

Products moved
to the clinic

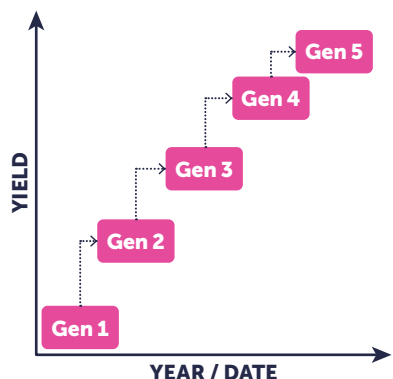
8

Innovation patents
processed

FLT201 (Gaucher disease type 1)

FLT190 (Fabry disease)

FLT180a (Hemophilia B)

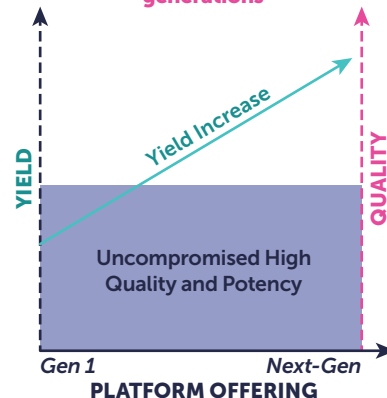


SEQUENTIAL PLATFORM ADVANCEMENT

100-Fold Improvement target

There remain major barriers to enhancing AAV production in insect and mammalian cells not evolved to function as AAV factories. Ascend is applying innovative engineering solutions to overcome these barriers with a goal of achieving 100-fold improvement in yield over the next 10 years.

We deliver yield scalability with no compromise of quality and potency across scale, capsids and platform generations



Ensuring longevity with:

- Standalone services through to programs from early- to late-stage
- Upstream and downstream process, formulation, and analytical development optimization
- Proprietary EpyQ™ AAV production system and triple transfection offered in house
- Investment in the most advanced starting materials (plasmids and cells) and media
- Novel assays to enhance programs and exceed regulatory requirements
- Unbiased screening and multi-omics studies

Aim higher

 **ascend**



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ADVANCED THERAPY MANUFACTURING SPECIALISTS