

Technical Article

Ramp Up Your AAV Production with the Xcite™ AAV Platform Technology

Lonza in Your Lab™

With the AAV market gaining momentum, gene therapy developers are under increasing pressure to advance their AAV-based therapeutics for clinical trials quickly, while managing the risks and the time-consuming steps of gene therapy drug development. Leveraging Lonza expertise and the same tools and technologies implemented for our platform process, you can balance risk and speed, while maintaining high AAV productivity. Xcite™ AAV platform technology is established based on our proprietary suspension HEK293 cell line and plasmid vectors, proven to enhance AAV production. These materials are now available for use in your own laboratory.

Introducing Xcite™ AAV platform technology

AAV-based therapies require a scalable GMP manufacturing platform that is robust and can support high titers during production. Traditionally AAV manufacturing for gene therapy entailed the use of labor-intensive adherent-based HEK293 processes not amenable to scale-up. Suspension adaptation of HEK293 cells has enabled more scalable processes, but remains time-consuming. Low AAV productivity and lack of platform processes continue to be key challenges driving AAV production cost up. In response to these challenges, Lonza has established a robust and scalable suspension manufacturing platform for AAV production.

At the heart our Xcite™ AAV platform process is our proprietary HEK293 Host Cell Line (5B8) and AAV Production Plasmids. The 5B8 cell line is a HEK293 suspension host cell line, selected and developed at Lonza for high AAV productivity. The 5B8 cell line is cultured in animal-component-free conditions and has demonstrated high expression and scalability for AAV production (Figure 1). Research and cGMP cell banks are available, under license, to take to your laboratory.

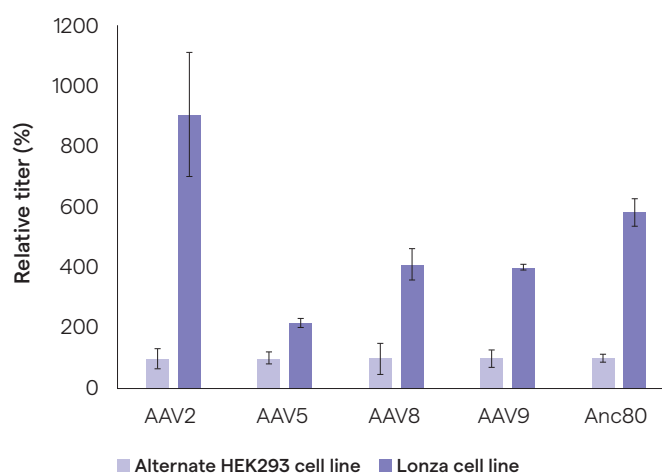


Figure 1

AAV-GFP Expression in Lonza HEK293 Cell Line versus alternate cell line. Anc80 is an *in-silico*-derived serotype (Zinn et al., Cell Reports, 2015).

The cell line productivity is further boosted by Lonza's proprietary Helper and RepCap plasmids (patent pending) across multiple AAV serotypes and genes of interest (GOIs) (Figure 2).

The 5B8 cell line and know-how for production of Lonza's Helper plasmid and Rep/Cap promoter are now also available to license for use in your laboratory or production facility.

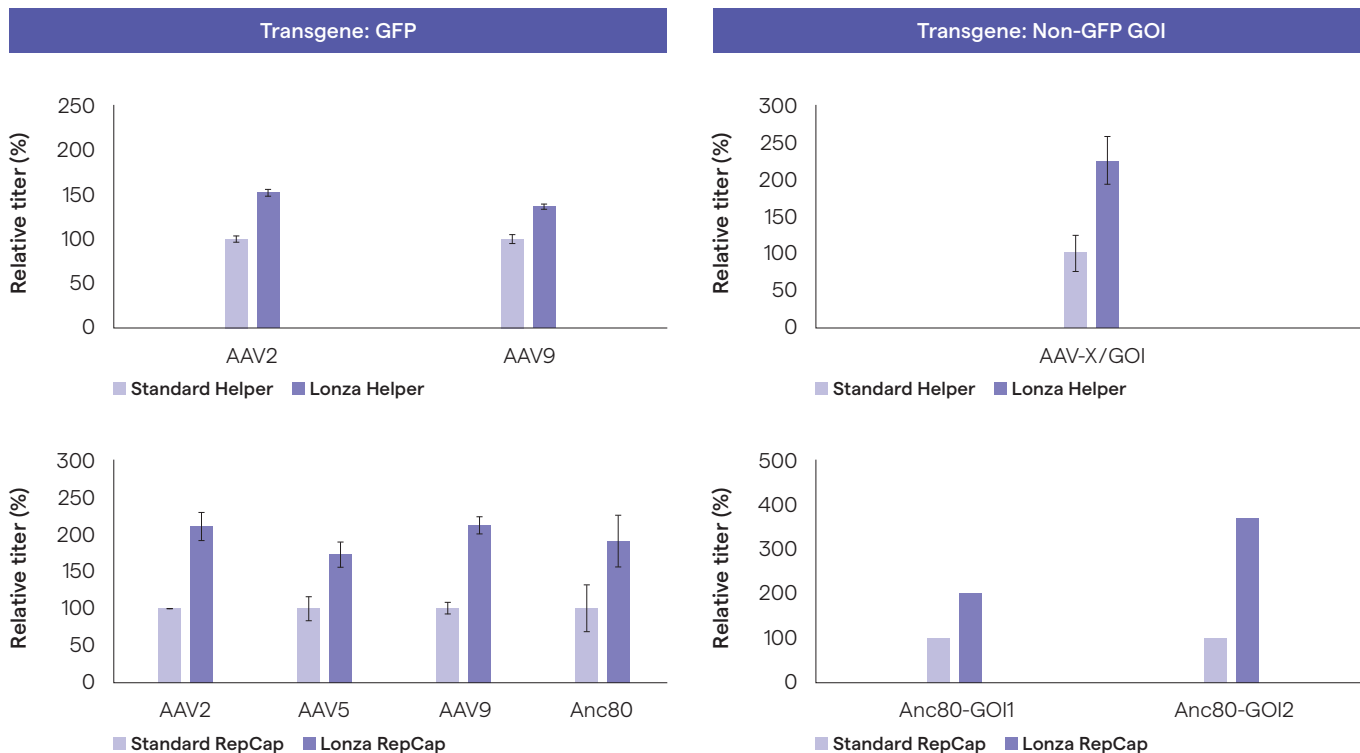


Figure 2

Superior performance of Lonza proprietary AAV production plasmids compared to standard plasmids in Lonza 5B8 cell line. Anc80 is an *in-silico*-derived serotype (Zinn et al., Cell Reports, 2015).

The Xcite™ AAV platform technology is now available to take to your laboratory

5B8 Production Host Cell Line

- Suspension HEK293 cell line
- High AAV productivity for multiple serotypes and GOIs
- Proven scalability for AAV production
- Research and cGMP cell banks available under license

AAV Production Plasmids

- Proprietary know-how on pHelper plasmid and promoter for balanced Rep/Cap expression
- Higher productivity and titers¹
- Optimized for multiple AAV serotypes and GOIs

¹ Lonza in-house data, comparison to standard commercially available plasmids.

To license our AAV platform components, email VVET@lonza.com

Lonza in Your Lab™

For more details and to discuss how Lonza's AAV Platform can ramp up your AAV production, contact us at vvet@lonza.com.



Scan QR code or click here to learn more about Lonza's Xcite™ AAV platform.

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