

# Viral gene therapy platforms for dermatology

## Introduction

Krystal Biotech is a clinical-stage gene therapy company using its patented Skin TARgeted Delivery (STAR-D) platform to develop effective and innovative treatments for rare skin diseases. They perform every stage of the manufacturing process in-house.



The company is leveraging the advantageous properties of type 1 Herpes simplex virus (HSV-1), including the virus's natural affinity for skin

cells, to develop a safe viral gene therapy platform adapted for dermatology. The engineered HSV-1-based system is suited for a topical route of administration, allowing for non-invasive therapeutic applications when treating the skin.

## User Commentary

*"The NucleoCounter® NC-200™ provides us with a much more reliable, consistent and traceable method of quantifying nuclei. We have also found that the NucleoCounter® NC-200™ is a great way to obtain reliable and accurate cell counts. Although this instrument was originally purchased for process development, all our R&D teams now use it for cell counting because it is fast, user-friendly and dependable. The NucleoCounter® NC-200™ is easily one of the most-used instruments in our laboratory."*

Lauren Regula, Leading Scientist, Krystal Biotech

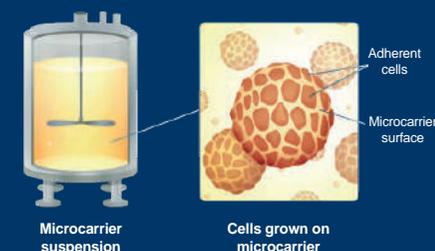
Learn more about Krystal Biotech at: <https://www.krystalbio.com/>

## Challenges

Krystal Biotech's initial products are directed to the treatment of monogenic and congenital skin diseases, including dystrophic epidermolysis bullosa (DEB) and autosomal recessive congenital ichthyosis (ARCI). In addition to Krystal's pioneering work developing novel treatments for rare skin diseases, the company is leveraging its expertise and proprietary gene therapy platform to explore new therapeutic approaches for additional dermatological indications, including non-monogenic diseases and diseases not caused by inherited genetic defects.

In-house manufacturing brings a number of unique opportunities and challenges. Obtaining daily cell counts during cell expansion and vector production for process development requires precise nuclei counts which are difficult to obtain manually. Krystal's main challenges therefore were to implement a reliable method of easily and accurately quantifying nuclei and to be able to institute rapid process improvements identified by the Process Development (PD) and Chemistry, Manufacturing and Controls (CMC) teams.

Bring unrivalled cell count precision and accuracy to your production pipeline



The NucleoCounter® NC-200™ brings you high precision data from microcarrier cell cultures in 5 minutes. You get consistent cell count and viability data, while avoiding harsh detachment reagents which may affect your results.



21 CFR Part 11-ready, the NucleoCounter® NC-200™ can be implemented throughout product workflow, from early-stage research to manufacturing and QC.