DATAHOW

Unlocking Gene Therapy with DataHowLab

Gene Therapy holds the promise to revolutionize medicine by managing the root cause of genetic diseases and unlocking the cure for otherwise untreatable conditions. To deliver on that promise, many challenges remain to reduce the development costs and make the therapies more affordable to patients.

A complex manufacturing process and high costs pose a challenge for development

A significant challenge in Gene Therapy processes is the complex, sequential nature of the steps involved, whereas CQAs are typically measured only at the conclusion of the process. As a result potential issues may only be detected at the end of the process resulting in many failed batches.

Furthermore, the high costs associated with these experiments leads to a limited availability of data. Scientists are challenged to develop these process within tight constraints.

DataHowLab enabling a consistent, effective digital development approach

DataHow's AI-based technologies are proving to be effective in delivering crucial development insights depsite these constraints, and creating a path to reliably and consistently developing gene therapies with a digital development approach.

The following highlights from industrial cases, illustrate the impact of DataHowLab on Gene Therapies:

Case 1: Assessing DataHowLabs ability to predict final CQAs with limited experimental data.

Case 2: Assessing DataHowLabs ability to generate insights accross each phase of a gene therapy process to support a more sequential control strategy.

Case 3: Assessing whether data from historical projects can be used to improve process insights for novel gene therapy developments.

Technologies in Focus

Hybrid Modeling

DataHow's hybrid models fuse a "mechanistic" backbone, which code known process dynamics, with data driven machine learning models to learn that which is unknown.

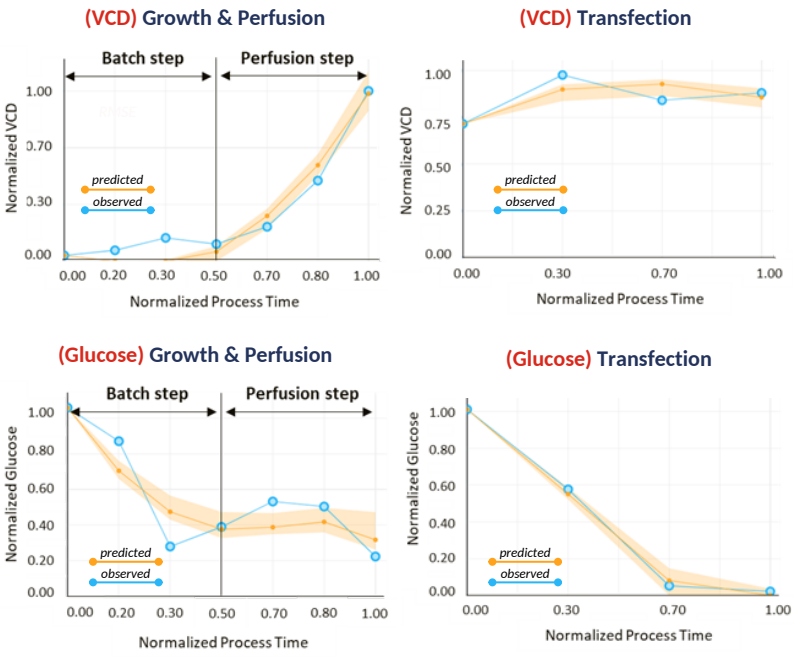
The result is a flexible modeling approach which provides deep process insights while requiring less data - an enabler of low cost accelerated development.

Transfer Learning

Transfer learning is a machine learning method that leverages data from historical processes to transfer knowledge horizontally to new projects.

This machine learning capability can be applied across molecules and across scales, offering further acceleration potential.

CASE 1 - Only 12 experiments need to accurately understand and predict final CQAs

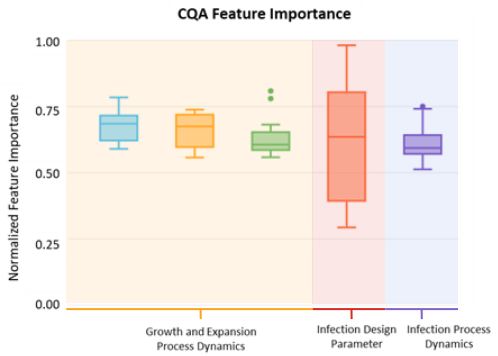


The analytical power of DataHowLab's hybrid models were able to confidently predict process CQAs with only **12 experiments.**

The models successfully predicted outcomes across multiple process stages—growth, perfusion, and transfection—demonstrating their flexibility and capability to analyze the process holistically.

DataHowLab equips scientists with the tools and technology to confidently predict gene therapy processes across all production phases while minimizing experimental effort, time, and cost.

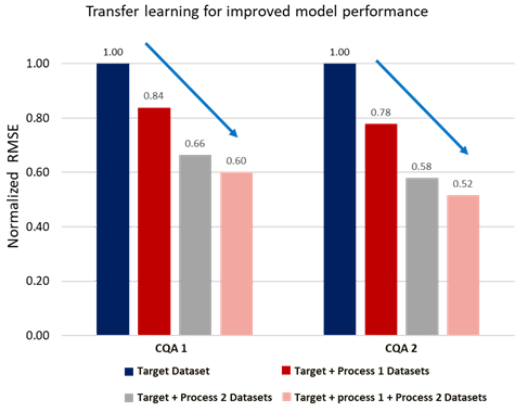
CASE 2 - Dynamic understanding and control of CQAs across all phases of production



DataHowLab not only evaluates the impact of process parameters on final CQAs, but can also interpret the relative impact of the key parameters on each CQA at each of phase of production, and evaluate their individual & combined effects.

DataHowLab enables the development of variable importance projections across each process step for each CQA. These provide key guidance in designing effective, phase-specific control strategies.

CASE 3 - Leverage historical process data to improve CQA prediction and reduce experimentation



Leveraging DataHowLabs transfer learning methodology, datasets from 2 historical GT processes (1 + 2) were included in order to transfer insights to the target therapy.

The results demonstrate that incorporating data from other Gene Therapy processes, in particular Process 2, can effectively increase insight decrease prediction errors.

This approach offers scientists the possibility to reduce experiments, costs, and development timelines for novel developments by leveraging historical data.

Consistent GT Development Approach

Reducing Development Costs

End-to-end Process Understanding

Historical Data as a Development Asset