Unlock the Promise of Gene Therapy with the Benchling **R&D** Cloud





Design, Develop, and Characterize Novel Gene Therapies on a Unified Digital Platform

# A Digital Solution for the New Age of Gene Therapy

Gene therapy stands at the forefront of the search for cures to severe genetic disorders. Technologies such as plasmid DNA, viral vectors, bacterial vectors, non-viral vectors, oncolytic viruses, CRISPR, ZFNs, and TALENs are giving R&D teams new ways to innovate in this research area. Modern gene therapy requires investigation of a wide variety of genes of interest, engineering of delivery vectors, and optimization of vector production processes. This necessitates a strong digital foundation to comprehensively characterize gene therapy candidates, maintain traceability across sequences, samples, and processes, and generate actionable insights to bring the best products into the clinic.

## Modern Gene Therapy R&D Requires Dynamic, Purpose-Built Software

Gene therapy R&D teams need a modern, powerful informatics platform that can help efficiently advance their unique research pipeline. An optimal solution provides the following tangible benefits to R&D organizations:

#### **Ensuring traceability**

Research teams investigate new genetic targets and evaluate the feasibility of complex approaches such as CRISPR, DNA delivery, and oncolytic viruses. These teams have to manage a wide range of genetic materials, delivery vectors, and biological samples, and capture data and experimental workflows with unique considerations. This demands software that can not only model the diverse range of samples used, but also improve traceability across samples.

#### Improving scientist productivity

Gene therapy R&D teams often use siloed point solutions, ELNs, or LIMS to support their research needs. However, as the teams grow in size and program complexity increases, traditional point solutions struggle to keep up. To boost productivity, gene therapy organizations need software that can fully unify complex gene therapy R&D, from plasmid design to bioprocessing to product characterization, so data capture can be standardized and tasks automated.

#### **Fostering collaboration**

Gene therapy R&D requires complex coordination among specialized teams focused on lead discovery, vector production, and analytical testing, all working in close collaboration. As a result, data needs to be captured in both unstructured and structured forms, and experimental context needs to be shared often and in a timely manner. This creates a clear need for software that can streamline collaboration and enable complete context transfer.

#### Providing access to key insights

Scientists generate extensive data throughout every stage of the gene therapy R&D lifecycle, and must regularly make critical optimization and selection decisions. To make optimal go/no-go decisions, teams need an informatics platform that connects every step of their workflow from lead discovery to viral production and analytical testing, helps aggregate data effortlessly, and provides key insights across the full gene therapy R&D pipeline. "What Benchling gives us most of all is speed. It allows us to process more [NGS] samples and generate that data more quickly so that we can make decisions faster."



Bob Gantzer Head of Lab Automation & Robotics



### Discover and Develop Breakthrough Gene Therapies on Benchling's Unified Informatics Solution

Benchling is a modern, unified, fully configurable, and easy-to-use cloud software application that adapts to the rapidly evolving needs of gene therapy R&D. It enables scientists to study gene editing and gene therapy technologies at scale — resulting in better traceability, enhanced productivity, intuitive collaboration, and contextual insights. This digital solution combines the capabilities of traditional ELN, LIMS, and molecular biology tools, while providing native support for gene therapy R&D. With Benchling, researchers creating cutting edge gene therapies can bring these breakthrough treatments to market faster.

#### Traceability

2x

increase in confidence in data quality and completeness

#### Collaboration

95%

improvement in cross-team data transfer and collection Insights

64%

decrease in time spent compiling data for analysis and reporting Informatics

4.5x

typical ROI realized by Benchling customers 4

### A Complete Solution for Gene Therapy R&D



#### Research

Accelerate Gene Therapy Research with Fit-For-Purpose Data Models, High Throughput and Collaborative Tools, and Access to Timely Insights

### Manage a centralized library of complex components of gene therapies

Build a library of all your samples, such as genetic materials, plasmids, endonucleases, and delivery vectors. Link each sample with each other with a fully configurable data model, and capture detailed properties in customizable schema fields. This helps centralize all sample information and improves sample traceability.

### Collaborate end-to-end across genetic engineering, vector design, and testing

Streamline collaboration across entire gene therapy R&D workstreams with a unified platform that helps maintain full context for each sample and experiment. Permissions and access controls further enable role-based collaboration across the entire R&D lifecycle. This helps scientists work more effectively with teammates.

### Increase throughput of sequence design and in-lab experimentation

Design and analyze CRISPR guides, plasmid sequences, and viral vectors at scale using more than a dozen cutting-edge in-silico tools with built-in wizards and bulk features. Record large volumes of experimental data against critical vector products with high-throughput capabilities. This helps scientists save time and reduce manual errors.

### Advance promising gene therapy lead candidates with greater visibility to all results

Standardize data and automate the capture of results into a central data warehouse. This allows easy aggregation of characterization results from diverse assays, and helps program leaders and executives ask new questions across studies and programs, leading to improved decision support for critical go / no go decisions.



#### Development

Ensure Efficient Development with Collaborative Workflow Management, Sample Handoffs, and Process Insights

### Centralize entire upstream and downstream processes on a unified platform

Build a library of all your samples, such as Map upstream and downstream steps used for viral vector production onto a single platform that enables the tracking of key process parameters and output metrics to maximize vector yield. Additionally, integrate with process automation to reduce the number of manual steps. This helps boost scientist productivity and production efficiency.

### Enable complex gene therapy product characterization and data handoffs

Ensure all relevant sample information is relayed accurately to analytical and in vivo teams with complete experimental context. Integrate with analytical instruments directly to automate results capture and reporting against in-process and final viral lots. This simplifies sample and data handoffs between teams, and centralizes all available results.

### Iterate vector production runs and scale up studies faster and more efficiently

Create and iterate through viral production runs with templatized stages and entries for recording each step. Capture large amounts of data in a structured manner with results tables and direct instrument integrations. This helps scientists set up studies quickly and execute experiments efficiently, without tedious, manual, and repetitive actions.

### Uncover key insights to optimize vector production processes and scale up

Create comprehensive characterization profiles and optimize process parameters with customizable visual dashboards. Trace the lineage of any sample with ready access to full sample history. This helps scientists gain greater access to data and process metrics, enabling R&D teams to optimize workflows and make scaleup decisions more easily.



"What Benchling has provided us is a single solution for multiple different problems, from sequence design and alignment to a centralized database. Our speed has doubled, communication has improved exponentially, and it's decreased scientist frustration."



Brenda K. Minesinger, PhD Principal Scientist



The Benchling R&D Cloud is Trusted by Leading Gene Therapy R&D Companies



Join over 200,000 scientists using Benchling to power life science R&D.

Request a Demo