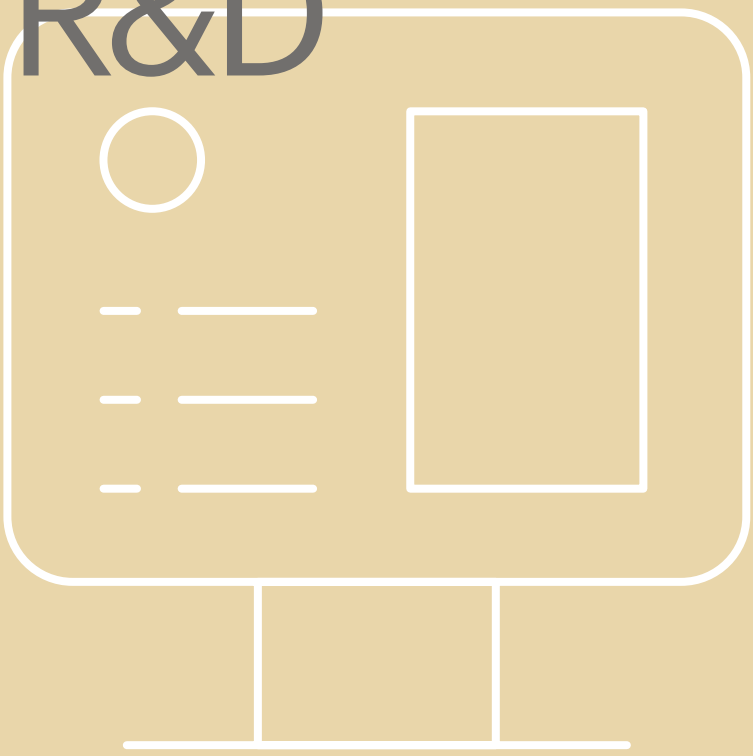
White Paper

# Buyer's Guide: Choosing an Informatics Platform for Gene Therapy R&D



An evaluation framework for assessing the critical features that separate a modern platform from legacy software

Benchling

# Choosing an Informatics Platform

Gene therapy marks a new era of medicine, bringing with it a new R&D paradigm where the product is a complex combination of genetic materials and viral or non-viral delivery vectors. As a result, conventional lab informatics platforms are not always optimal for gene therapy R&D.

To help gene therapy R&D teams find an informatics platform that will efficiently advance their unique pipeline, we've created an evaluation framework that highlights the critical features that separate a modern, powerful platform from legacy software and overcomplicated spreadsheets.

## Complexities in gene therapy R&D

### Vector Design

As an organization expands the number of therapeutic targets,internal research teams are tasked with designing and managing a wide range of genetic materials and delivery vectors.

### Process Optimization

Turning a complex product, such as a gene therapy,into a highly reproducible manufacturing process requires extensive scale-up studies during process development.

### Data Handoffs

Establishing a close partnership between product characterization and process development to support complex product characterization needs is essential to successful gene therapy development.

Having worked with hundreds of gene therapy companies, Benchling knows what it takes to be successful. In this buyer's guide, we dive into the key capabilities that have enabled our gene therapy customers to accelerate their R&D activities. Here's a brief overview:

**Does it support your science?**
Consider how the platform represents and links the entities you use every day, such as genes, plasmids, and delivery vectors, as well as the workflows you commonly complete, such as vector design, process scale-up, and assay development. Will it be easy to configure for your workflow or require extensive custom development?

**Will it improve productivity?**
Assess if the software standardizes and automates tasks, such as sequence uniqueness checks and data capture, enables bulk actions of repetitive tasks, such as plasmid design, and integrates and automates as many upstream and downstream vector production steps as possible. Does the software minimize timeconsuming tasks and integrate the entire process?

**Does it foster collaboration?**
From plasmid development through product characterization, the software should enable team members to simultaneously work on different aspects of a particular task, make it easy for team members to find and use data generated by someone else, and facilitate efficient data handoffs between research, development, and analytical teams. Can you easily communicate and share workflows and data with team members and other departments?

**Does it offer access to key insights?**
Whether you need to evaluate genotoxicity and off target effects, optimize the conditions of vector expansion, or limit chromatography load times, your informatics platform should be able to pull together all the key data from different teams and parts of your workflow into easy-tounderstand reports and dashboards. Can you easily visualize the data you need the way you want?

# Does it support your science?

## What is the critical need?

Gene therapy R&D teams are constantly investigating a variety of complex approaches such as CRISPR gene editing, DNA delivery with viral and non-viral vectors, and oncolytic viruses — each with unique considerations for capturing data and experimental workflows. Without a unified informatics platform that can properly model this diversity, information gets stored in siloed systems, resulting in key insights remaining hidden, collaboration struggles, and missed opportunities.

## Features and capabilities to look for in a solution

### Models your gene therapy processes out of the box or requires minimal customization

A flexible registry system allows you to easily create libraries of plasmids, vectors, and cell lines and track interconnected samples with sequence-level intelligence.

### Maps the relationships between the different plasmids and vectors you work with and traces the lineage of your samples

A centralized platform delivers full connectivity so you can trace sequence-based information, quickly find viral analytics results, and auto-link experiments and inventory management.

### Inherent flexibility that supports a variety of genetic engineering and vector design processes as your needs evolve

Easy integration and codeless configurations (and re-configurations) enable quick and easy adaptations for process and portfolio changes, making it constantly future-ready.

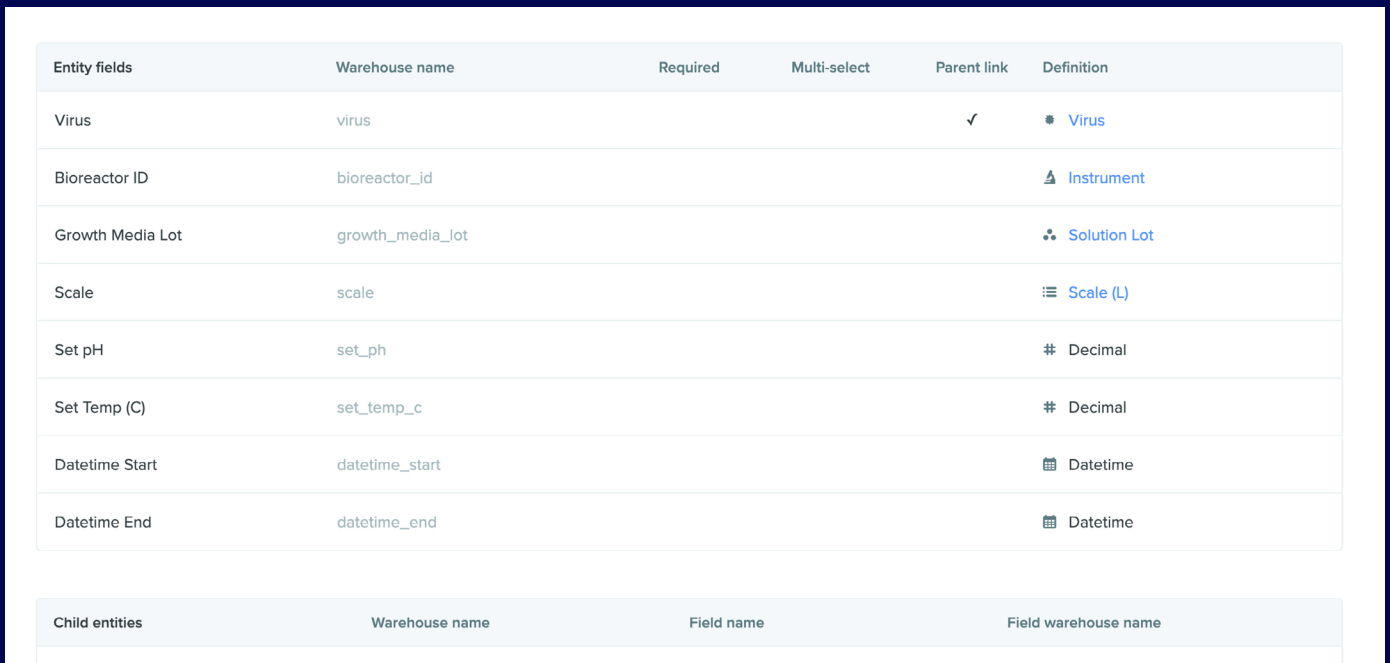
### Customization capabilities to capture relevant information for specific vectors and lead candidates

Custom schema fields enable you to track key metrics and properties, such as editing efficiency, transfection efficiency, and genotoxic sequences.

## Benchling supports gene therapy R&D

Benchling's comprehensive suite of applications, such as Molecular Biology and flexible Registry and Inventory applications, are designed to fit any type of gene therapy R&D. This enables teams to customize data capture, design CRISPR guides and vectors, and map the relationships between samples, sequences, and relevant data. Additionally, Benchling's codeless configuration and unified data layer can integrate with any lab instrument and software, making it quick and easy for teams to adapt the software to new processes. Benchling is always future-ready.

Learn More



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| Growth Media Lot | growth_media_lot | | | | Solution Lot |
| Scale | scale | | | | Scale (L) |
| Set pH | set_ph | | | | Decimal |
| Set Temp (C) | set_temp_c | | | | Decimal |
| Datetime Start | datetime_start | | | | Datetime |
| Datetime End | datetime_end | | | | Datetime |

| Child entities | Warehouse name | Field name | Field warehouse name |
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# Will it improve productivity?

## What is the critical need?

Gene therapy R&D teams often use electronic lab notebooks (ELNs) and traditional laboratory information management systems (LIMS) to manage experiments and workflows. However, these tools are often disconnected from each other and other digital tools, resulting in poor usability, potentially costly errors, and an inability to automate several key repetitive tasks, including sequence creation and data capture against sequences and samples. To boost productivity, gene therapy organizations need software that can fully unify complex R&D processes—from plasmid design to bioprocessing to product characterization—so that as many tasks as possible can be standardized and automated.

## Features and capabilities to look for in a solution

### Standardizes data capture across users, teams, and studies

Customizable templates help standardize DNA sequence entry, in vitro and in vivo assay recording, and viral analytics results, ultimately ensuring reproducibility and compliance.

### Reduces the number of manual and repetitive tasks in plasmid design and vector production

Bulk action capabilities for plasmid design and integrations with semi-automated and automated bioreactors can boost vector production efficiency and minimize time-consuming, manual activities.

### Enables you to quickly set up your experiments for vector development, lead discovery and preclinical testing

Automatically documenting experiments with templatized notebook entries linking to registry and inventory records can help your team find, design, and set up experiments as quickly as possible.

### Unifies your entire R&D workflow from target identification through bioprocessing and product characterization

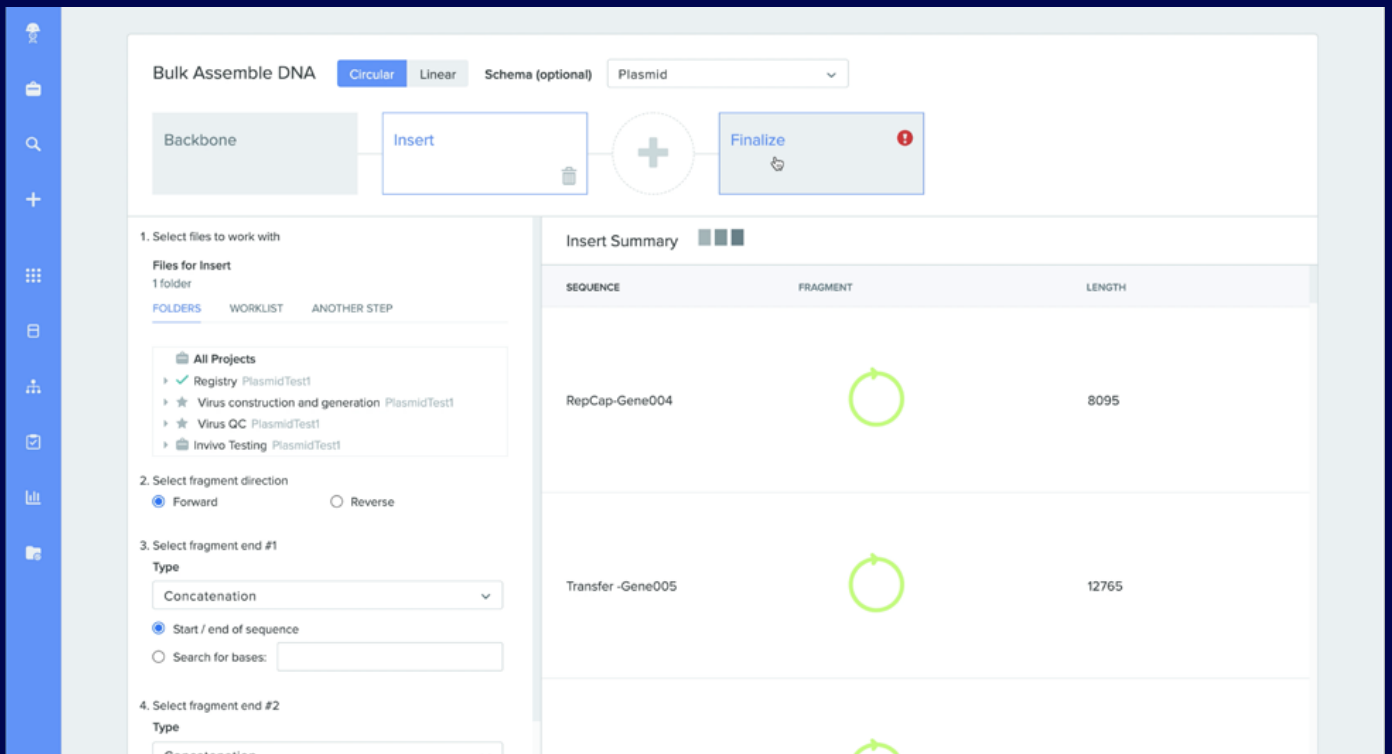
A centralized platform enables you to quickly find vector-specific results and track process outputs such as transfection efficiency, viability, purity, potency and yield from multiple runs.

## Boost productivity in gene therapy R&D with Benchling

The average scientist saves 1 day week when they switch to Benchling.

Benchling's codeless configuration, user-friendly interface, customizable dashboards and templates, and automation features allow gene therapy companies to integrate every step of gene therapy R&D onto a single platform. Benchling can be leveraged to track key process metrics and standardize and automate data capture and experimental documentation, ultimately improving research efficiency.

Learn More



# Does it foster collaboration?

## Why is this a critical need?

Gene therapy companies need software solutions capable of facilitating collaboration between discovery, development, vector production, and analytical teams, meaning the software needs to be flexible enough to handle unstructured data (such as experiment notes) and structured data (such as numerical results). Currently, R&D teams record data in a variety of locations and formats, such as paper notebooks and disconnected ELNs for unstructured data and LIMS for structured data. However, separate information systems like these make collaboration between teams difficult, as it can lead to incomplete context transfer and inefficient data handoffs.

## Features and capabilities to look for in a solution

### It helps minimize the number of different software systems R&D teams have to use

By integrating with your instruments and your existing digital ecosystem, a best-in-class gene therapy informatics platform can replace multiple pieces of legacy software with a solution tailor made for processes such as vector production and product characterization.

### Ensures data traceability and accessibility for every lead candidate and gene therapy product

By automatically linking assay results to specific batches of in-process materials and final products, a unified system can deliver complete traceability.

### Enables seamless sample and data handoffs between teams

A centralized data repository for research, development, and analytical teams can streamline context sharing while handing off samples or data at every step of characterization for source materials, isolates, in-process vectors, and final gene therapy products.

### Tools to help team members collaborate more effectively

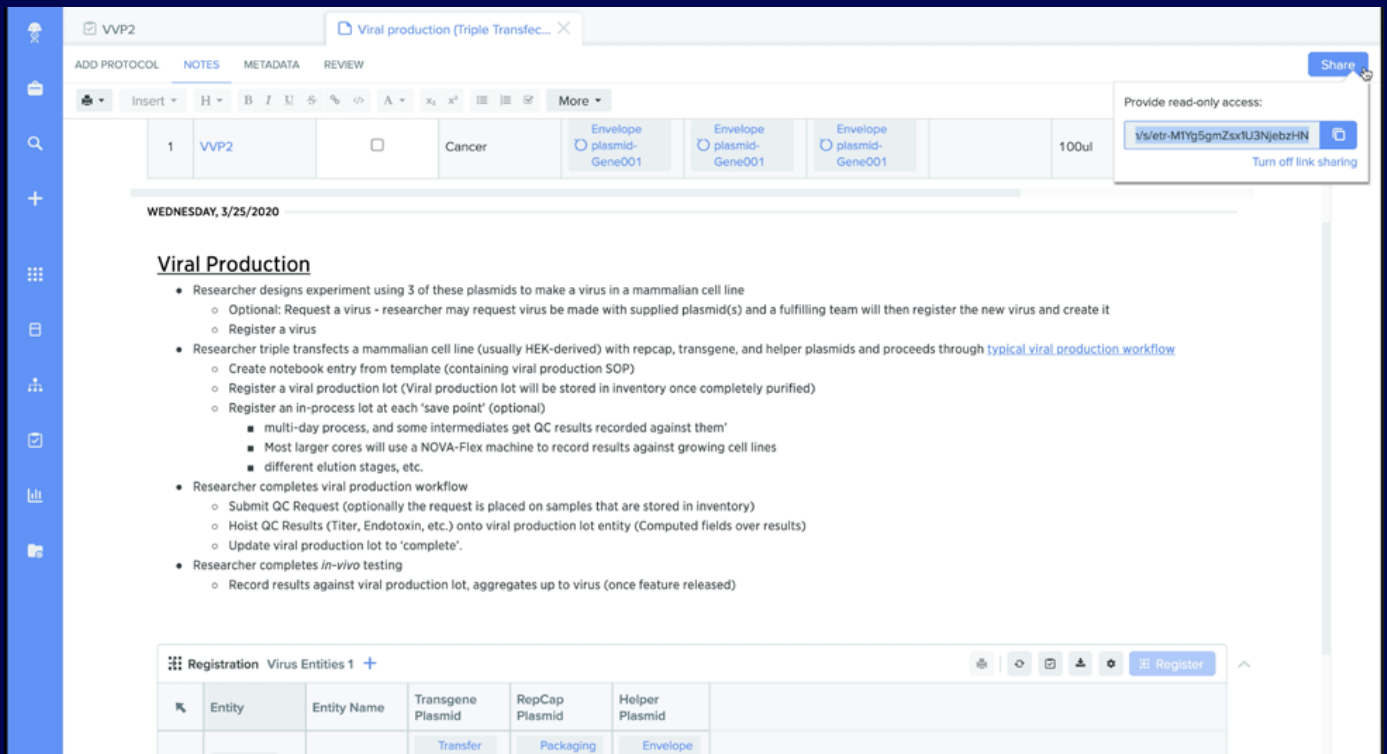
Permissions and access controls where you can assign specific tasks with detailed vector information enable better coordination of sample chain of custody, as well as improve communication, across analytical and process development teams.

## Benchling facilitates effective collaboration in gene therapy R&D

With Benchling, you get a single software solution for your entire R&D workflow. You can seamlessly connect upstream and downstream teams—from those working on plasmid design through vector production optimization through gene therapy product characterization—all on the same centralized, standardized system.

With our software, you can leverage 10+ molecular biology tools in one collaborative environment, so you can track all of your sequence edits through a complete version history and work together with shared libraries to eliminate duplicate efforts.

Learn More



# Does it offer access to key insights?

**What is the critical need?**

Gene therapy R&D creates massive and meaningful data at every stage. This data needs to be monitored, mined, reported, and acted upon, but information is often siloed due to the use of disconnected software solutions. As a result, scientists curate data from different sources and report key findings across their organization using PDF reports or PowerPoint presentations, which are laborious, error-prone, and difficult to reference after the fact.

To make optimal go/no-go decisions, gene therapy companies need an informatics platform that connects lead candidates to discovery workflows, viral production, and analytical data; related samples, plasmids, and DNA sequences; and input and output metrics for gaining operational insights into the full gene therapy R&D workflow.

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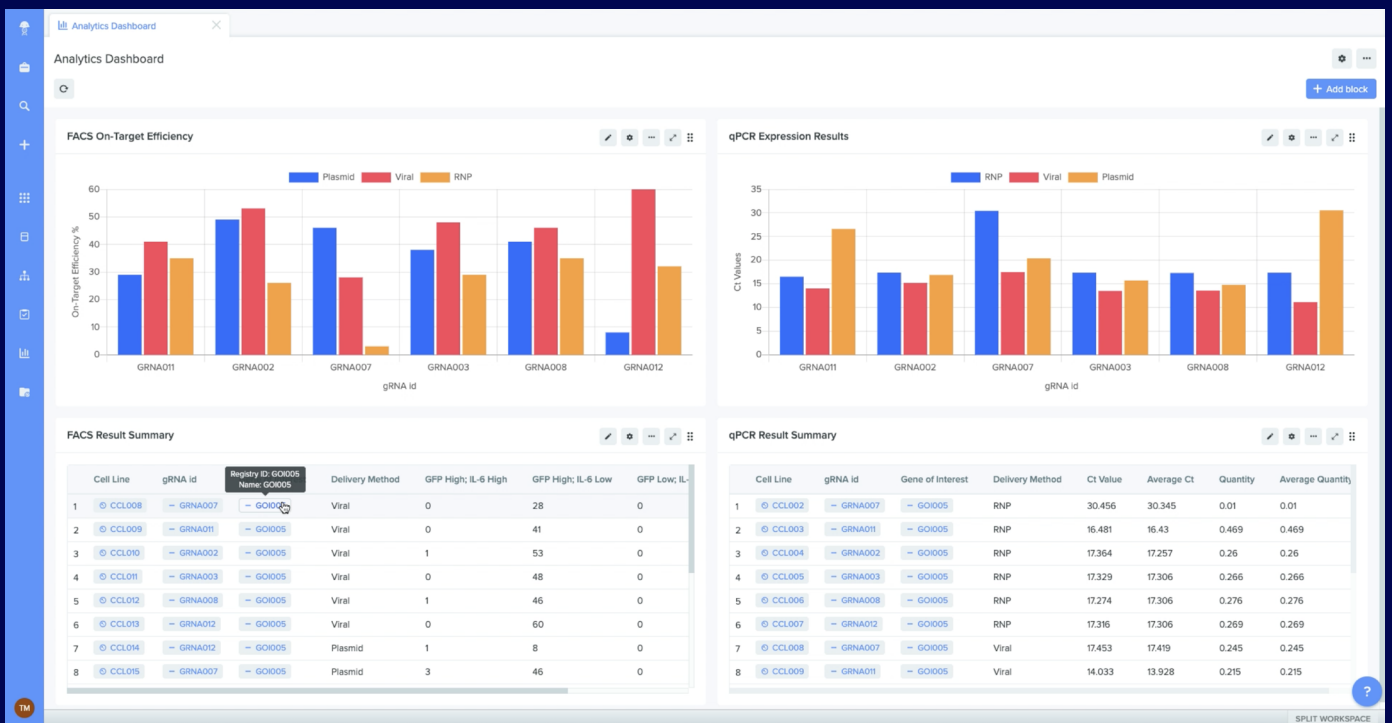
## Benchling helps teams gain scientific insights in gene therapy R&D

With Benchling, you get centralized upstream, downstream, and operational data, with complete sample traceability and customizable dashboards. This can help you easily find the samples and results that any sequence has led to and view the related processes and results the way you need to. Your company's data is always at your fingertips. With greater access to data and the ability to analyze complete R&D workflows with your choice of process metrics, R&D teams can gain deep insights to optimize upstream and downstream vector production processes and make critical scientific decisions.

Learn More



# Conclusion

Patients and families worldwide are living with incurable, undruggable diseases, but gene therapies offer them a ray of hope. With our evaluation framework, you can find purpose-built informatics platforms that help you turn these hopes into a reality, avoiding software that can't manage the complexities of gene therapy R&D and that hinders your progress to get breakthrough therapeutics to market and patients.

Benchling is a future-ready, fully configurable, and user-friendly platform that is purpose-built for the rapidly evolving needs of gene therapy and gene editing R&D.

**Support your science**
Our flexible platform, comprehensive suite of molecular biology tools, and codeless configuration can adapt to any gene therapy workflow—from viral and non-viral vectors to CRISPR R&D.

**Improve productivity**
Our software is easy to use, while automating and minimizing time-consuming tasks associated with plasmid design, data collection, and vector production.

**Foster collaboration**
Coordinate sample and data handoffs and streamline communication across research, analytical, and process development teams on our platform that can automatically interlink your R&D workflows from plasmid development through release testing.

**Gain access to insights**
In-process, product release, and investigational assay results are all centralized and viewable with customizable dashboards so you can gain a comprehensive understanding of your gene therapy pipeline and the related processes.

**Support Your Science**

- Unified platform with fully integrated molecular biology, ELN, registry, and inventory management systems
- Easy, flexible configuration (e.g., codeless configuration)
- Flexible registry system proven to model diverse entities (e.g., DNA sequences, vectors, plasmids)
- Vector design capabilities
- Customizable schema fields

**Improve productivity**

- Easy to navigate and intuitive interface
- Customizable templates and results table for standardized data capture
- Automated data capture
- Bulk action capabilities (e.g., bulk plasmid design)
- Quick experimental documentation

**Foster collaboration**

- Permissions and access controls
- Automated data linking
- Integration with lab instruments and 3rd party software
- Support the needs of upstream and downstream teams so all R&D data is centralized on a single platform

**Gain access to insights**

- Centralized data warehouse
- Customizable dashboards for viewing process and operational metrics
- Variety of graphs and plots to visualize titer levels, qPCR readouts, assay results, and other viral analytics data

With Benchling, you can get started with the system of the future today to accelerate gene therapy R&D and bring more breakthrough therapies to market faster. Request your custom demo to see how Benchling can transform gene therapy R&D at your organization.

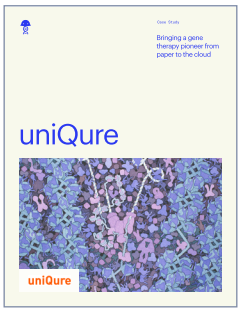
Request a Demo

# Additional Content

**Webpage**

Benchling for Gene Therapy and Gene Editing R&D

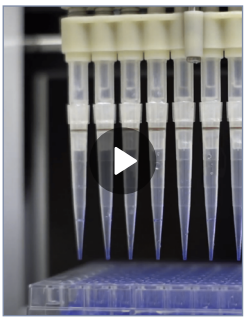
Learn More




**Case Study**

Bringing a Gene Therapy Pioneer from Paper to the Cloud

Read the Case Study



**Video**

Benchling Powers Automation and NGS at Beam Therapeutics

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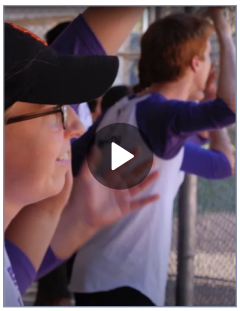

**Video**

The Jennifer Doudna Lab Knocks CRISPR Out of the Park

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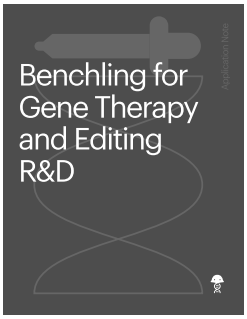


**Application Note**

Benchling for Gene Therapy and Gene Editing R&D

Download Application Note