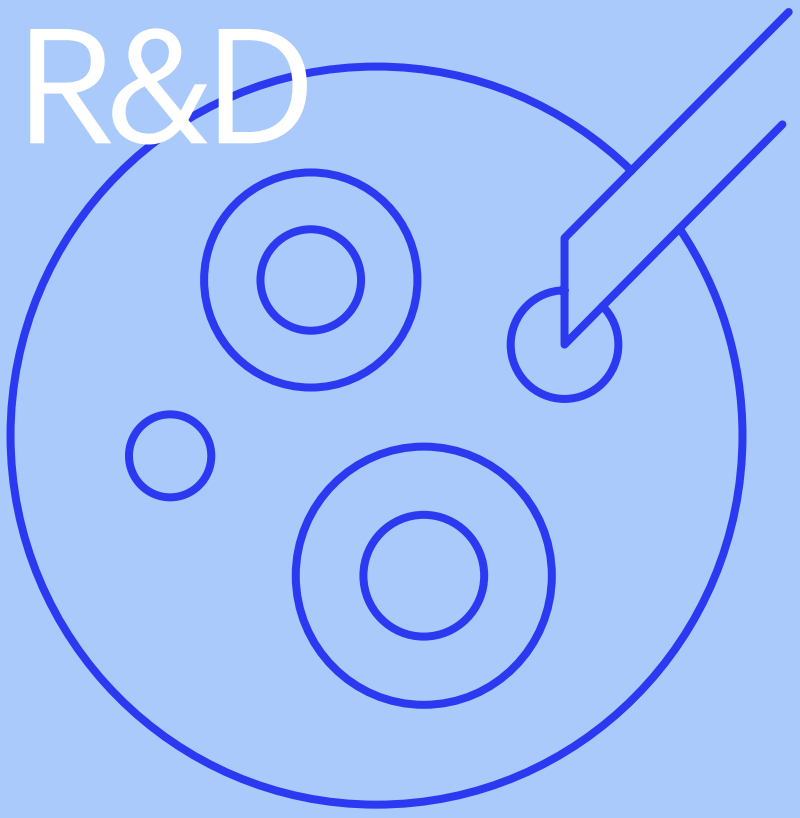
Solution Guide

Six Essential Digital Capabilities to Accelerate Cell and Gene Therapy R&D



A guide to the most important software capabilities required to stay at the cutting edge of cell and gene therapy innovation

Benchling

Unlocking the promise of cell and gene therapies

There is an undeniable change sweeping over the fields of cell and gene therapy. After decades of research and the first drug approvals just a few years ago, these promising drug modalities have entered a new era of industrialization. In the race to address unmet medical needs with more targeted biopharmaceuticals, companies have formed specialized R&D teams and units dedicated to these disciplines. With this focus comes new challenges: how to operationalize increasingly larger and more complex data, how to collaborate efficiently across specialized functions, and how to reduce cycle time between key milestones.

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The value of a strong digital foundation

Successful cell and gene therapy companies are embracing the role that digital solutions play in catalyzing innovation. Every aspect of R&D has the potential to be part of a digital transformation, but this has been harder than it seems. R&D software hasn't kept pace with the rapid innovation seen across cell and gene therapy. Companies in this field have had to rely on siloed point solutions, spreadsheets, and custom software to stitch their workflows together, and still find this inadequate to trace their candidates through the R&D lifecycle.

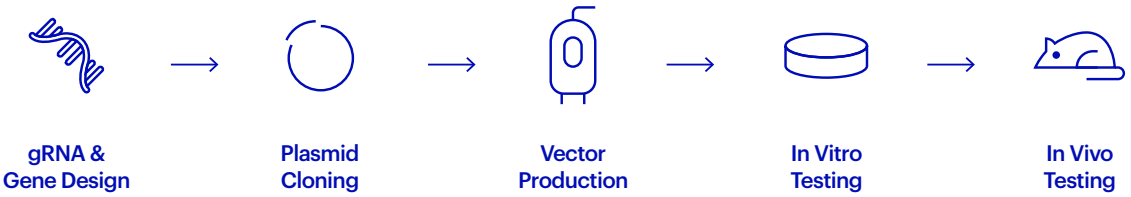
Here, we share essential digital capabilities that will transform your cell and gene therapy R&D programs. Each one on its own brings value, but companies committed to digital transformation will seek to bring many of these types of capabilities into their digital foundation. Benchling has supported hundreds of innovative cell and gene therapy companies, and all of the capabilities depicted here are essential elements of the Benchling R&D Cloud.

Mapping The essential capabilities across your cell and gene therapy R&D workflows

Every company focused on cell and gene therapies pursues their own differentiated scientific strategy, leading to unique workflows that span gene editing, construct design, bioprocessing, in vitro and in vivo testing. These workflows often change over time as R&D teams mature their science or choose new directions. It thus becomes critical to ensure that your digital foundation can support the breadth and complexity of modern cell and gene therapy across the full R&D lifecycle. Benchling has supported hundreds of cell and gene therapy companies as they've progressed their programs from early discovery to IND and beyond. The workflows below represent high level abstractions of how R&D is often structured, and we map the essential capabilities to where you frequently encounter them.

Representative Cell Therapy R&D Workflow

Representative Gene Therapy R&D Workflow



Capability 1

Configurable sample tracking to capture the full history of cell and gene therapies

Why It's Essential
Configuring a digital model of your science is foundational to your R&D workflows. By relating building blocks of your R&D such as plasmid sequences, scFv (VH/VL) sequences, viral vectors, and donor cell lines, you end up with a structured database of your unique process that forms the basis of data capture, process management, and insights.

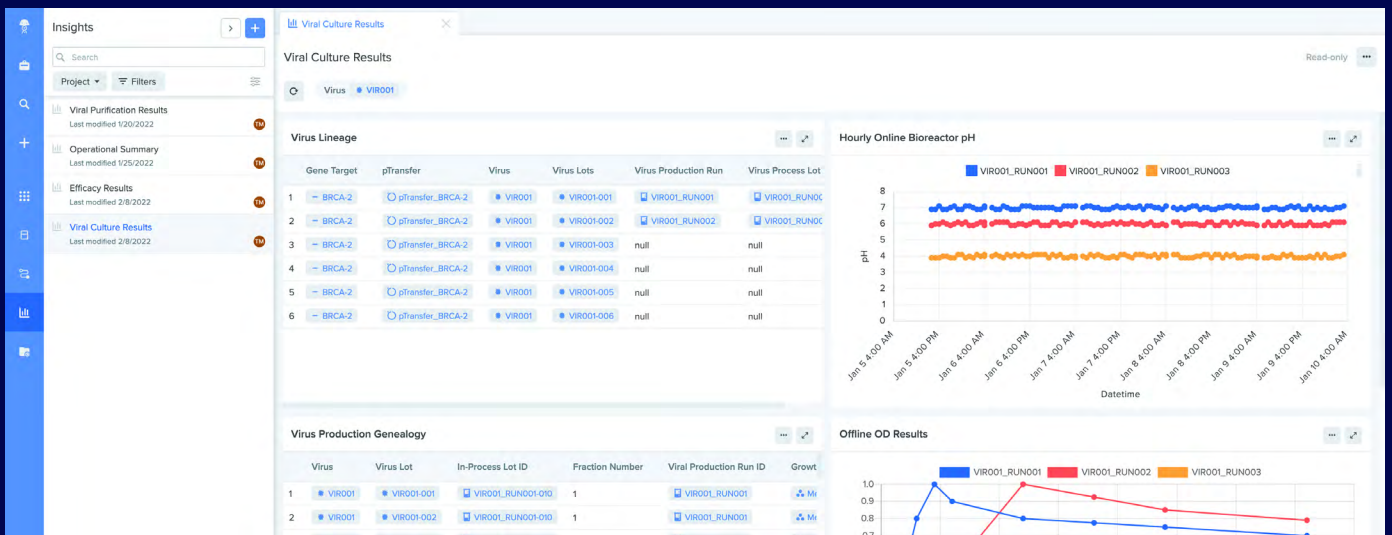
Why It's Often Difficult
Most R&D software has limited or no ability to create tailored, biologically-focused data models. In many cases, those that do provide this capability consider this a customization that takes considerable time and expense to maintain over time.

What You Gain
With configurable models that align to your science, each sample you collect aggregates within this structure. It forms the foundation for more effective search, process management, and data analysis, ultimately saving you time and accelerating the path to your next milestone.

Benchling's Customer Experience team meets with every new customer to understand their science, and map it to best practices learned from hundreds of cell and gene therapy implementations. These form the foundation of their data collection templates, process flows, and Insights dashboards. Modifications of your data model can be performed with codeless configuration, helping teams keep their digital foundation in sync with their science.

Here, an example is shown of the modeling of a virus in Benchling. It includes key elements of how the virus is produced, such as instrument identification, media selection, and operating parameters, all within a configurable user interface. As data is collected against this data model, insights can be derived across different viruses and production lots to identify and optimize the best candidates.

Entity fields	Warehouse name	Required	Multi-select	Parent link	Definition
Virus	virus			✓	Virus
Bioreactor ID	bioreactor_id				Instrument
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



Capability 2

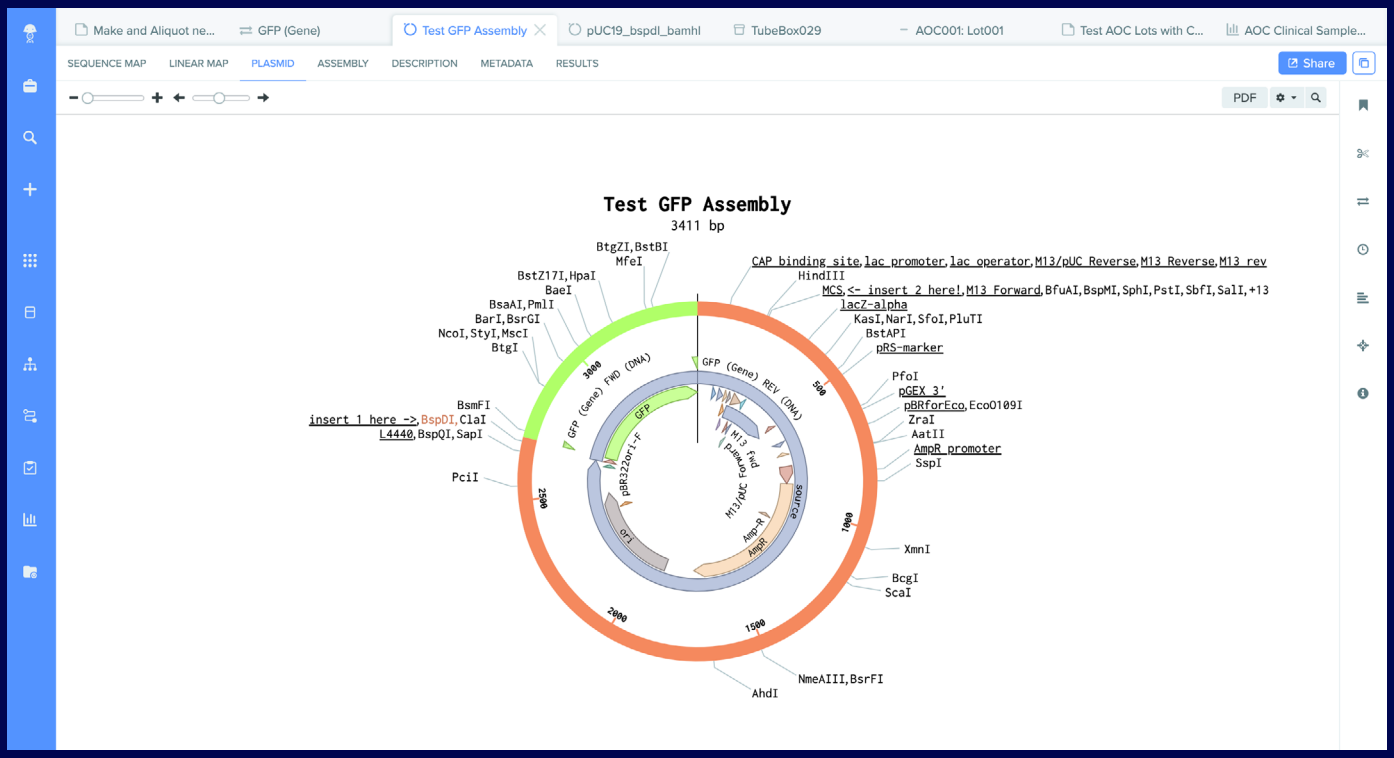
High-throughput sequence design and analysis with integrated uniqueness checking

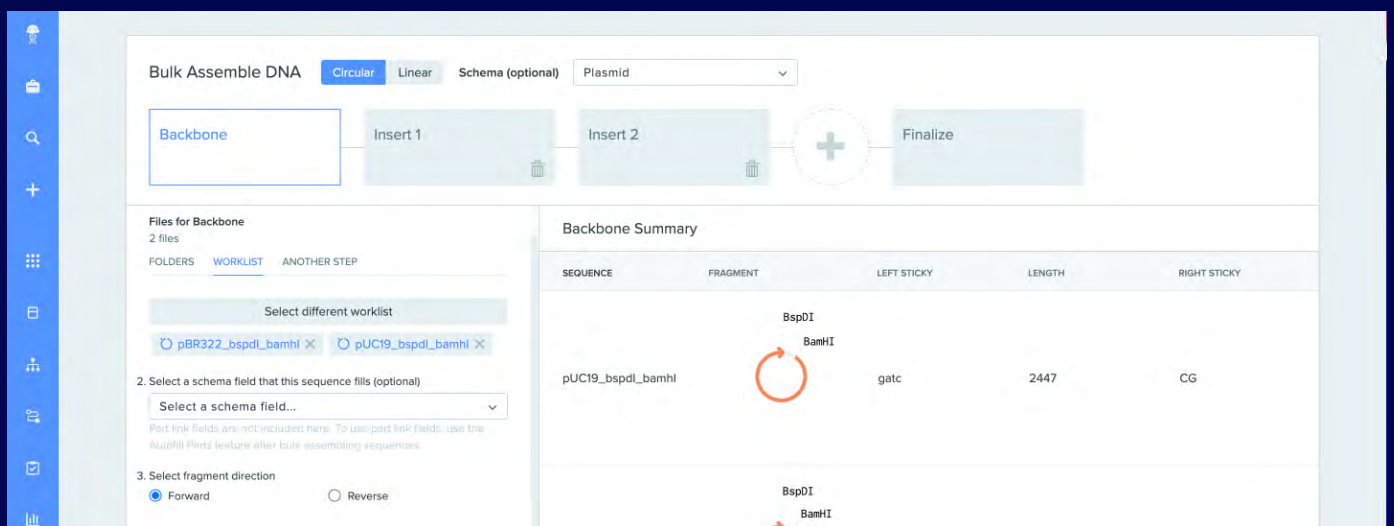
Why It's Essential
Cell and gene R&D programs frequently require creation of plasmid and sequence libraries composed of varying types of biological entities (e.g. genes of interest, scFv, promoters, ITRs). It is critical to be able to produce these plasmid designs at scale, and directly incorporate these into an intelligent registration system that will confirm their uniqueness.

Why It's Often Difficult
Most of the first-generation sequence design tools lack any kind of bulk assembly capabilities. Since these tools are typically on a different platform as the experiment and sample registration systems, it often requires manual transfer of designs through manual export or copy/paste steps.

What You Gain
Having high throughput assembly tools helps biology teams create plasmid and sequence libraries faster and more efficiently. The resulting outputs flow directly into the registration database with full traceability and annotation to the component sequences, helping teams centralize and standardize their data collection against these entities. With an intelligent registration system that checks for uniqueness, the underlying database is kept clean. Scientists can quickly understand if their plasmid has been designed before, and can save time by using existing supplies.

Benchling's bulk assembly tool offers an automated path to sequence creation for cell and gene therapy R&D. Combinations of genetic parts (e.g. promoters, genes, ITRs, resistance markers) can be automatically combined according to the set configurable parameters. The resulting library can then be bulk-added to the sample registry, generating full traceability across all parts of every sequence in the library.





Capability 3

Sequence awareness to automatically identify DNA and plasmid features

Why It's Essential

Modern cell and gene therapy companies design and produce high volumes of sequence-based constructs, such as genes, promoters, resistance markers, and inverted terminal repeat (ITR) sequences. As these sequences are designed into plasmids and vectors for delivery, it is critical to be able to identify every constitutive part of the vector. Having these parts automatically identified and associated within the sequence saves time, improves design efficiency, reduces human error, and brings traceability to the entire R&D process.

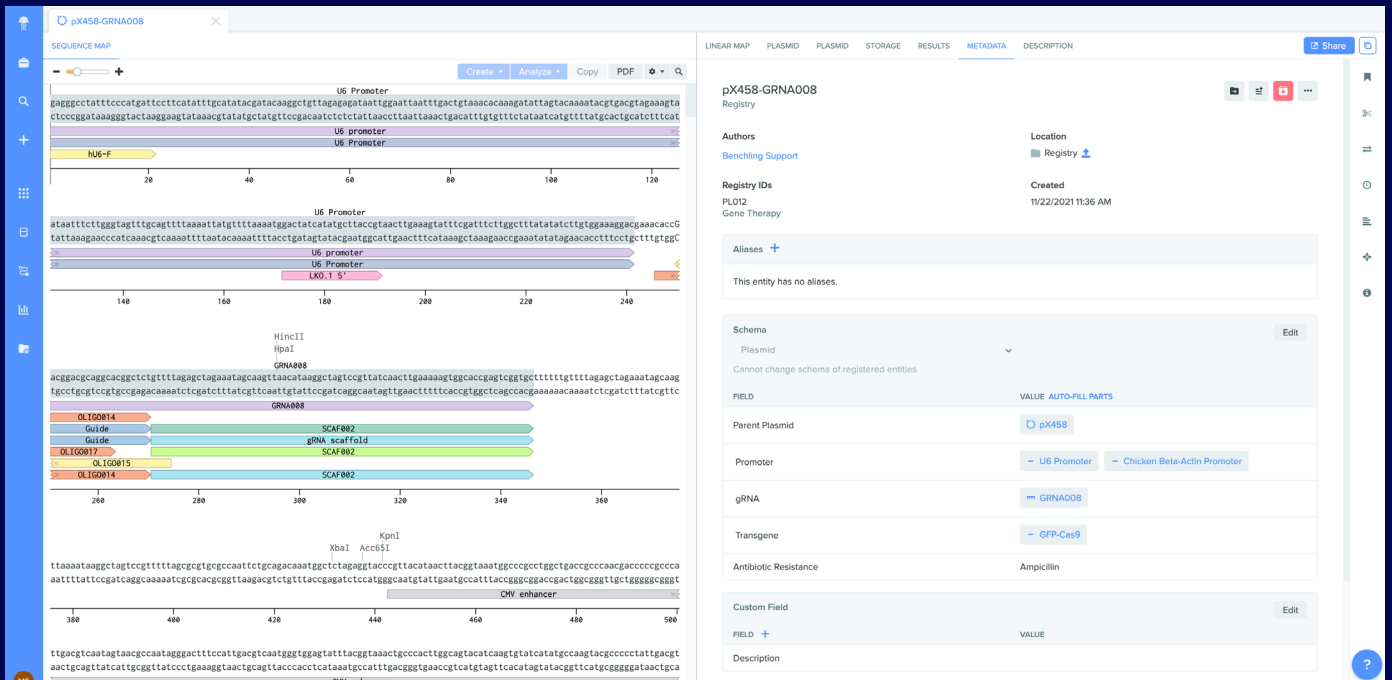
Why It's Often Difficult

Most software tools are not 'sequence aware'. When sequences are tracked in spreadsheets, ELNs, or LIMS, there is no means to detect which features are present. Stand-alone molecular design tools offer some ability to annotate sequence regions, but this context is frequently lost as the sequences are manually moved over into separate registration or experimental tracking systems.

What You Gain

When you work in an informatics system that automatically recognizes, annotates, and links key sequence parts, your teams will be able to trace samples throughout the entire R&D process. Design teams will save time by not having to manually append part identifiers across hundreds to thousands of entities. Vector production, in vitro, and in vivo teams will be able to readily identify the lineage of the samples they're working with, while gaining the ability to quickly search, filter, and analyze across these individual parts. This helps cell and gene therapy teams make more efficient design and development decisions, leading to increased R&D output.

Benchling's 'Auto-Fill Part Fields' capability automatically recognizes sequences for constitutive parts found in plasmids, such as genes, ITRs, resistance markers, and promoters, and creates structured links to them in the Benchling Registry. This saves considerable time during the design process. Additionally, this increases productivity across all downstream R&D teams that work with these samples, as they will gain the ability to search, filter, and analyze samples based on their composite genetic parts.



Capability 4

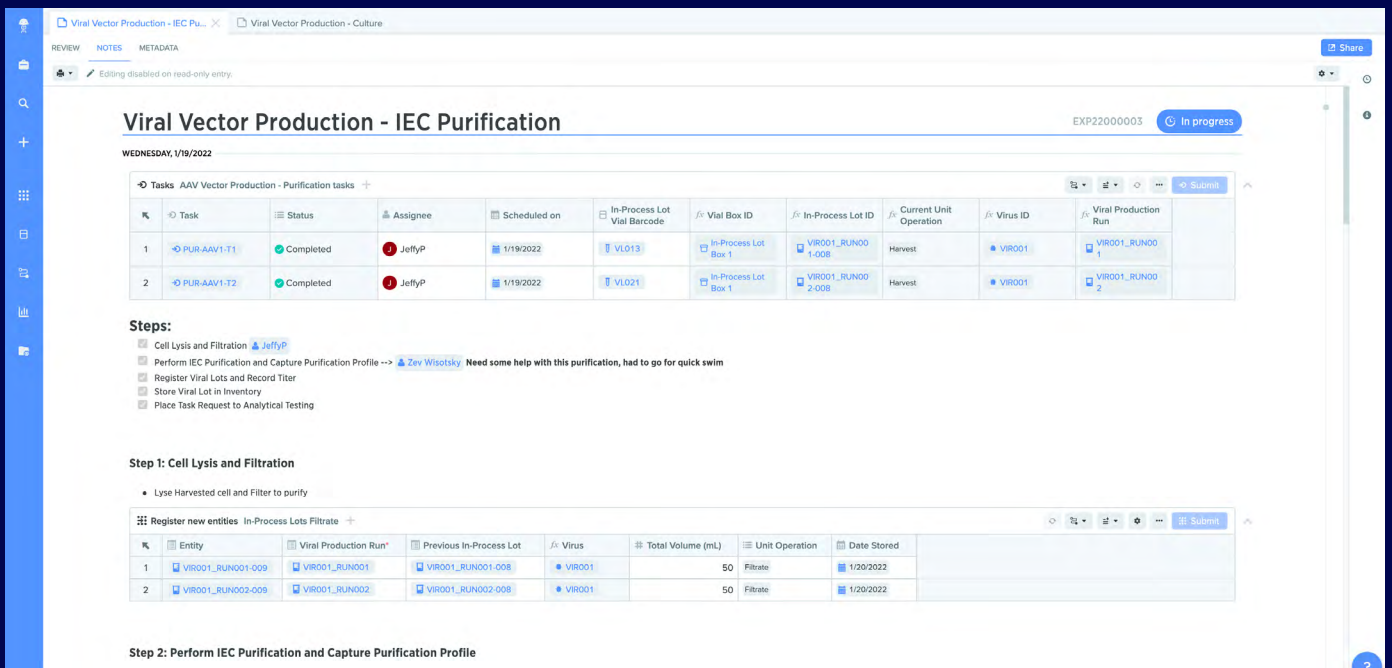
Configurable templates to enforce standard processes across teams

Why It's Essential
Cell and gene therapy R&D produces high volumes of complex data. It is critical that this data is collected in a structured, consistent manner, even early in the discovery process. Templates provide a clear path for moving experimental and process data, and the corresponding samples, into a structured data repository.

Why It's Often Difficult
Most labs have experimental protocols and SOPs documented, but they tend to be in spreadsheets and office documents. These formats have no connection to the data repositories your company likely operates from. Basic ELN and LIMS systems might be able to connect the protocols to the data captured for an experiment or process, but they typically lack the ability to perform essential data centralization tasks, such as registering samples, pulling from freezer inventory, and adding assay results to a structured database.

What You Gain
With configurable data collection templates, you gain the ability to control the input of data into a centralized location. For cell and gene therapy companies, templates can facilitate the registration of plasmid designs and preps, the addition of physical aliquots into inventory, the characterization of viral preps, and the structuring of in vitro and in vivo data into a single source of truth across R&D teams.

In Benchling's R&D Cloud, templates can be configured that align to key steps in the cell and gene therapy workflow, from guide and gene design, to plasmid cloning, vector production, and in vitro / in vivo testing. In addition to the experimental methods and protocols, Benchling templates contain registration, transfer, and results tables to automatically ingest samples and data into a central database.



Capability 5

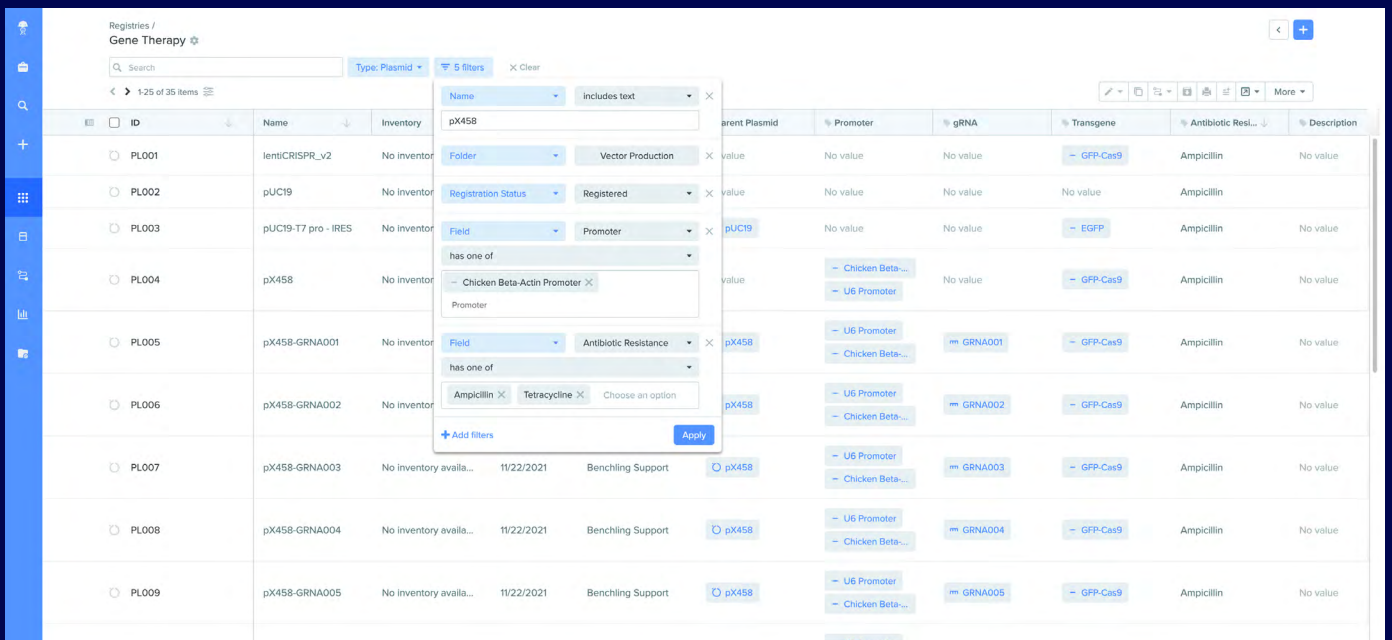
Advanced searches across samples and experiments

Why It's Essential
Cell and gene therapies comprise multiple sequence parts, and specialized R&D teams help progress these products from design to production and testing. Scientists frequently need the ability to search not just across their own work, but across the work of their team, program, or entire company. They require the granularity to refine these searches down to specific sequence domains of the products they are innovating.

Why It's Often Difficult
While search tools are prevalent in almost every application, they are frequently limited by how these applications are used and available search parameters. For example, you can search the contents of a spreadsheet but not across every spreadsheet in the company. Or you can search for lab notebook entries created in a certain data range, but you can't refine the filter to only look at entries containing a particular transgene plasmid.

What You Gain
Powerful search capabilities help teams access samples, inventory, and data faster, leading to improved scientist productivity and user experience. When you have global search that span entries, samples, and sequences, you can access the data in question much faster and more efficiently. When the search function extends across process steps, it empowers R&D teams to work more collaboratively together.

Benchling's global search bar is embedded across the R&D Cloud interface, providing an easy and efficient way to access data. Users can quickly search for any entity type in their company's configured data model (e.g. gene, plasmid, virus) and further refine by entity filters (e.g. viruses made by using a specified packaging plasmid). The search results contain the list of matching entities as well as other pertinent information (e.g. inventory status, other sequence-based parts in the virus, team member that created it).



Capability 6

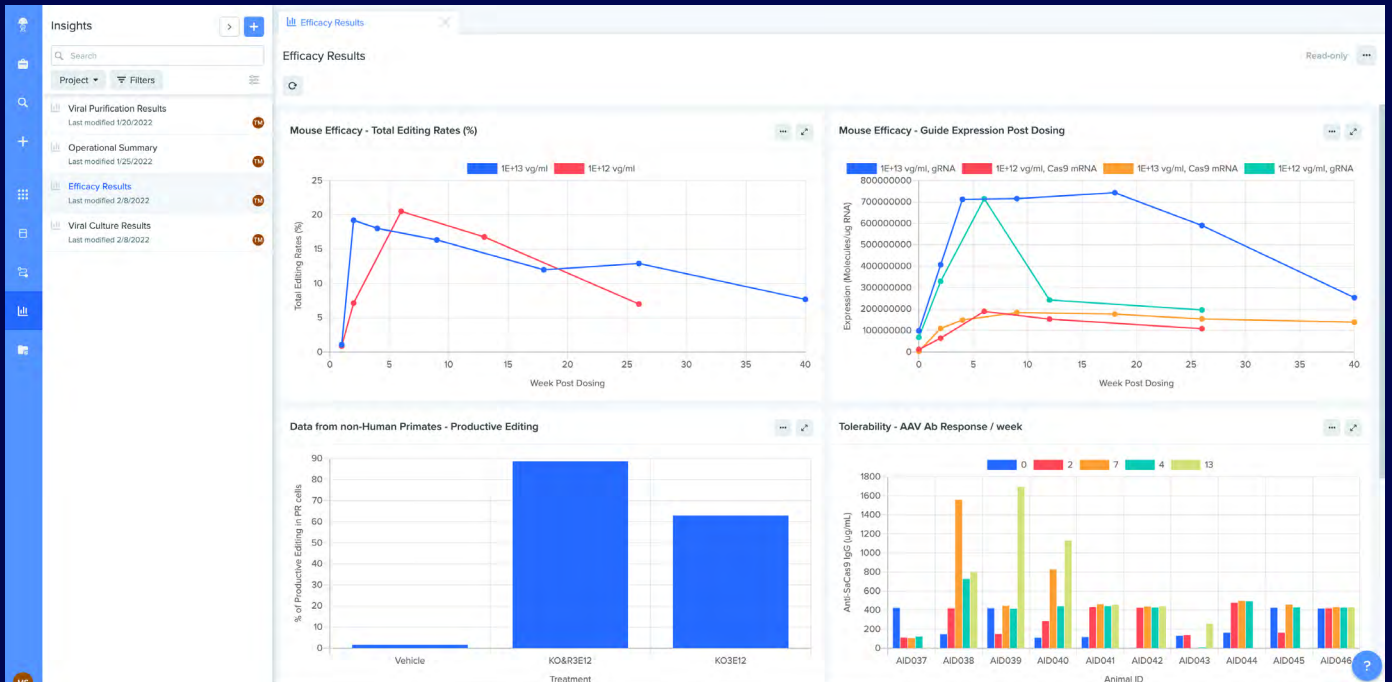
Insights across experiments, programs, and processes from a central data warehouse

Why It's Essential
The complexity of cell and gene therapies multiplies quickly with the portfolio of candidates being evaluated, the number of teams involved, and the number of steps in the overall process. R&D teams require the ability to ask granular questions from their data that cut across these layers of complexity. These precise queries are what help companies improve the velocity of their R&D output and reach milestones faster.

Why It's Often Difficult
There are numerous software applications that will plot and analyze data, from basic spreadsheets to scientific and statistical analysis packages, but this is rarely the bottleneck in cell and gene therapy R&D. The more frequently encountered challenge is running queries that require joined data from siloed applications and data stores. R&D scientists and data scientists alike often get pulled into manual, time-consuming data gathering steps to answer even basic questions that cut across teams and programs.

What You Gain
When your ability to query against structured data is directly coupled to your central source of truth for R&D, you gain greater access to scientific and operational insights. The data collected across programs becomes interoperable and sees a greater likelihood of reuse, instead of staying siloed and unmined.

Results captured in Benchling reside in a structured data warehouse that, when joined with sample and process lineage in the registry, form a complete foundation for running targeted queries against this data. Using reports, plots, and dashboards, as well as underlying SQL query tools, scientists can visualize trends, outliers, and patterns in their data. For cell and gene therapy applications, these queries cover the range of scientific insights (e.g. 'What media conditions led to the highest concentration of viral vector production?') and operational insights (e.g. 'How many cell expansion requests are we processing per month with the added staff?).



Benchling solutions for cell and gene therapy R&D teams

Packaged digital solutions for your cell and gene therapy applications
Benchling offers packaged solutions that map to essential functions found in cell and gene therapy R&D. All built on the Benchling R&D Cloud, these solutions extend Benchling's capabilities with data models, configurations, templates, and dashboards that maximize your time to value.

Delivered by scientific experts on our Customer Experience team
You won't have to explain what a plasmid is to our implementation team. All of them are steeped in science, with advanced degrees and bench experience, and bring the experience of working with hundreds of cell and gene therapy companies of all sizes.

Join a community of Benchling users across the world
Benchling supports over 700 customers across the world, from brand new startups to global leaders. Many of these companies are focused on cell and gene therapies, as well as other advanced therapeutic approaches. Additionally, we're supporting the next generation of scientists with over 200,000 users across 7,000+ academic research institutions.

Over 200 cell and gene therapy companies are innovating on the Benchling R&D Cloud

Join over 200,000 scientists using Benchling to power their biotech R&D. Visit benchling.com/request-demo to learn more.

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