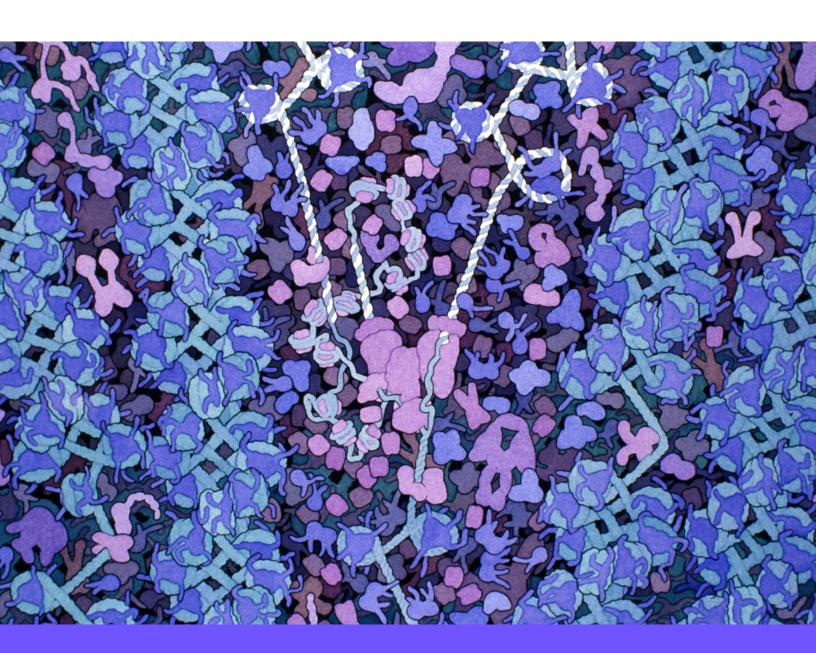


**CASE STUDY** 

# Bringing a gene therapy pioneer from paper to the cloud



### **GOAL**

# Bringing a gene therapy pioneer from paper to the cloud

Having originated the first approved gene therapy in 2012, uniQure is a leading pioneer in the field. By leveraging their modular and validated technology platform, uniQure is rapidly advancing a pipeline of AAV-based gene therapies to treat patients with hemophilia, Huntington's disease, and other severe genetic diseases.

### **COMPANY PROFILE**

# of employees: 250–1000 Industry: Biopharma HQ: Amsterdam

### **UNIQURE TEAMS USING BENCHLING**

Research Analytical Vector Process development development development

### **CHALLENGES**

Paper-based workflows made it difficult to track results and streamline experiment execution.

As an international company with worksites in Amsterdam and Lexington, Massachusetts, uniQure found it challenging to share data and collaborate across research sites.

A lack of standardized Notebook templates led to nonstandard experimental workflows and impeded quality control.

#### **KEY BENEFITS**

End-to-end tracking for gene therapy workflows

International collaboration — even while working remotely

Improved efficiency and enthusiastic adoption.



"It feels good to have something automated with just a single click, where before it took half an hour. As more people are getting familiar with the software, we're saving even more time."



Tim Hoogervorst, Technician, uniQure



## Key Benefits

End-to-end tracking for gene therapy workflows

Benchling automatically creates a complete history of every experiment. Scientists can track from vectors, to tissue samples, to viral batches and experimental results.

International collaboration — even while working remotely

Having a digital system enables internationally distributed researchers to collaborate and discover data much more easily – even when working from home due to the COVID-19 pandemic.

Improved efficiency and enthusiastic adoption.

Benchling Notebook templates massively improve scientist efficiency. 100% of the scientists licensed to use Benchling use the Notebook every day.

#### **APPLICATIONS USED**







Notebook

Registry

Inventory



# Benchling Solutions

## Using interconnected data to learn more from each experiment

- uniQure tracks vectors and stores critical data about them in the Benchling Registry, while linking them to downstream entities
  such as cell lines
- Benchling protocols standardize procedures and data entry, making it easier for scientists to analyze their experiments.
- uniQure's analytical and product development teams use Benchling to track every sample in their freezers, linking location data directly to results readouts. Scientists can easily quickly critical samples for continued experimentation.

### Improving R&D transparency and collaboration

- ✓ By transitioning from a paper-based system to Benchling, uniQure now has a cloud-based system where scientists and other employees can find their data more easily.
- uniQure scientists can track and access each other's work in real-time despite being on opposite sides of the Atlantic Ocean. This shortens the time to critical R&D decisions and ensures organizational alignment.
- As the COVID-19 pandemic has forced some uniQure scientists to work from home, they've been able to stay connected, design experiments, and analyze data to maintain project progress.

### Supercharging scientist productivity with an intuitive solution

- A long list of small improvements has had a significant impact on day-to-day productivity. For example, Benchling Templates have built-in calculations. Instead of typing away at a calculator, scientists spend their time analyzing results.
- Automatic audit trails and straightforward Notebook review processes ensure regulatory compliance and cut down on the time managers spend signing off on experiments.
- Through weekly configuration meetings and hands-on user training sessions from the Benchling Customer Experience team, uniQure seamlessly transitioned from a paper-centric culture to a digital one. 100% of the scientists licensed to use Benchling do so daily.



