Case Study

# Alnylam Puts Sites and Patients First with IQVIA Technologies Investigator Site Portal

*Six-year partnership plays pivotal role in rare disease trials*

## Challenge

In rare disease research, every patient is precious. That's why, when Alnylam Pharmaceuticals began development of medicines for rare diseases with few or no treatment options, the company's leaders knew they needed to design clinical trials that kept patient well-being and disease burden at the forefront.

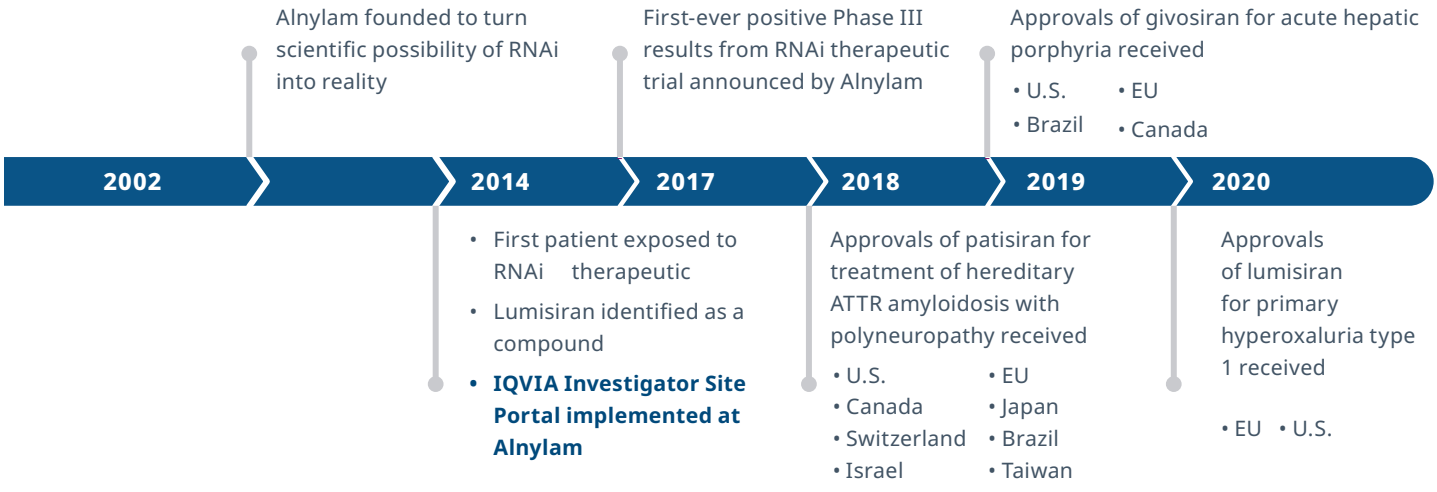
Alnylam also understood that rare disease patients find hope for the future in their specialists — the principal investigators and affiliated clinical research sites dedicated to finding new treatments and cures. Program leaders sought a solution that would maximize collaboration, communication, and transparency with sites and contract research organizations (CROs) — understanding that, as a young company developing an entirely new class of medicines on its innovative RNAi platform, building trusted relationships with investigators and key opinion leaders would be vital to long-term success.

## Solution

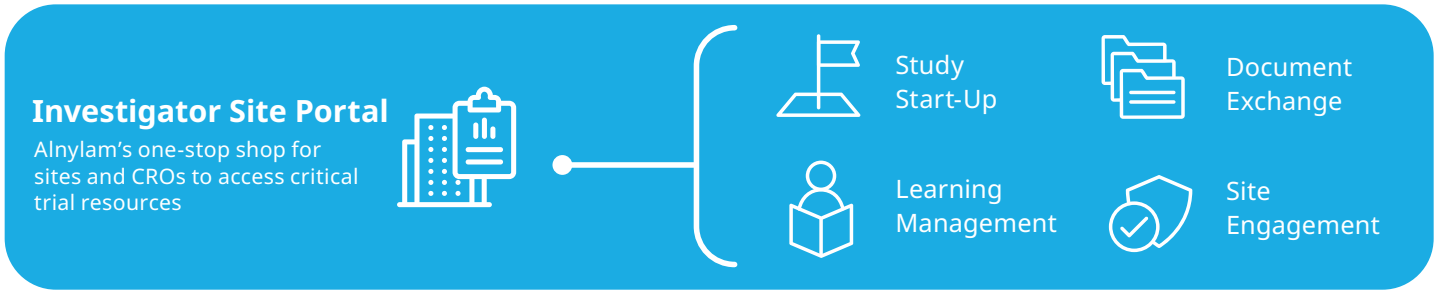
As Alnylam's first clinical program began in 2014, the study team saw emerging site-facing technologies as a foundation for those trusted relationships, starting with a purpose-built communication channel and easy-to-use study conduct tools to support a complex protocol.

Alnylam partnered with IQVIA Technologies to utilize the Investigator Site Portal and an experienced services team in its earliest human trials. Previously known as Trial Networks and DrugDev Spark, the Investigator Site Portal includes robust modules for site activation, learning management, document exchange and safety notifications, all tied together with market-leading site communication and engagement capabilities.

**Six-Year Partnership in Rare Disease Trials**



**A Collaborative Trial Solution**




Alnylam relentlessly pursued innovation and technology to improve trial conduct and site relationships in every trial since. As its development programs grew, so did its reputation for fostering connected relationships with investigators and sites. Study teams use the Investigator Site Portal in a common way across all trials, leveraging the full spectrum of "site-first" digital tools available to be applied to specific study challenges. Examples include:

### SITE NEEDS ASSESSMENT DURING COVID-19 PANDEMIC

During the early months of the COVID-19 pandemic, Alnylam leveraged the Investigator Site Portal to assess and respond to site needs. Study leaders used the electronic survey and email blast capabilities to get answers directly from sites, without needing to burden Clinical Research Associates (CRAs). With response rates averaging greater than 85%, surveys informed Alnylam on a broad range of ideas to keep studies on track:

- Interest in and need for home nursing services
- Interest in performing certain assessments such as a 6-minute walk test in open-air spaces in the community rather than at the site
- Resourcing issues that could lead to a stop or delay in screening/enrollment
- On-site monitoring status

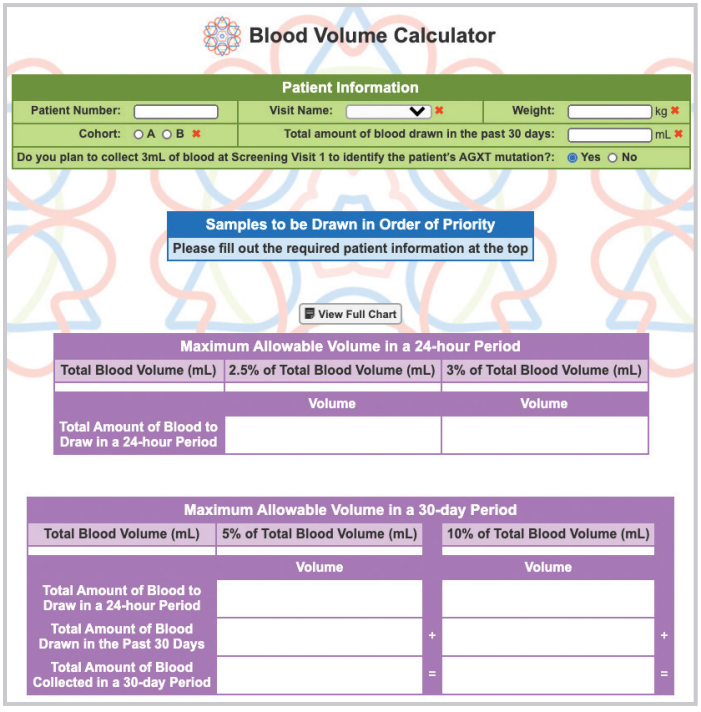
Alnylam included the opportunity for open-ended feedback and was able to respond to requests such as:

- Providing N95 masks for patient visits
- Protocol allowances for delays in scheduling
- More time to screen patients
- Flexibility with dosing windows

### CALCULATOR TO KEEP YOUNG PATIENTS SAFE

In one trial, frequent blood draws created a challenging situation for the study's youngest patients that are limited by daily and monthly weight-based blood volume collections. To ensure patient safety and reduce the risk of over-collection, IQVIA Technologies built Alnylam a customized Blood Volume Calculator to determine the priority of samples to be drawn and alert site staff when the limit had been reached based on patient weight. This eliminated a tedious site task of calculating blood limit values, significantly reduced protocol deviations, and most importantly, kept young patients safe.

Site coordinators and CRAs appreciated how the customized tool reduced risk in this highly complex program.



> **The DrugDev system is very convenient to use. Information is readily available in one single system (work documents, videos). This provides an overview of the current patient status... which has been confirmed to be appreciated by the site.**

> **The Blood Volume Calculator is very helpful to check or clarify the conditional samples to be obtained. The print output function supports sites in a convenient way to have printed evidence for source support.**

> **A very flexible system, tailor-made study-specific with easy access. A nice to have on such a complex program.**

### INSIGHTS INTO PATIENT RECRUITMENT

With patient enrollment integral to rare disease trials, insights into the recruitment process are extremely important to Alnylam. The Pre-Identification (Pre-ID) Log in the IQVIA Investigator Site Portal makes pre-screening and screening information entered by sites visible to relevant CRAs and study teams in real-time.

- Patient history and enrollment information is entered as the patient progresses through screening
- History of updates is available for review and annotation
- Protected Health Information (PHI) is maintained

The Pre-ID Log gives Clinical Trial Managers early indicators of the sites with the largest pool of eligible patients, enabling them to maximize the results of recruitment efforts.

### ENROLLMENT UPDATES

Alnylam uses weekly updates to keep all sites abreast of enrollment status across the study and leverages leaderboards to generate friendly competition. The tool has proven to inspire and motivate sites to stay focused on recruiting patients. Awarding virtual badges has brought positive attention to those meeting their goals.Site coordinators and CRAs appreciated how the customized tool reduced risk in this highly complex program.

Beyond motivational badges, the information shared keeps sites informed of important ongoing enrollment in studies that could affect their patients.

*We used the pre-screening logs in one of our trials to spark friendly competition among sites through the Activity Challenge. The top three sites with the most activity, measured by the amount of pre-screening information entered, could choose a rare disease charity of their choice to which Alnylam donated on their behalf."*

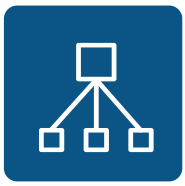
*— Clinical Trial Specialist, Alnylam*

### THE SITE EXPERIENCE

The Investigator Site Portal delivers the data quality, timeliness, and transparency that is critical when interacting with research sites and contract research organizations (CROs). Through a single URL, sites enter a familiar, intuitive environment designed on four key tenants:

**EASE-OF-USE**

The SaaS technology delivered immediate benefits to sites with virtually no onboarding or training required. Sites were able to eliminate time-consuming manual processes, streamline workflows, and leverage the same training and templates across multiple studies.



**PREDICTABILITY**

The Portal provides sites with a common environment across all studies, with a consistent user interface and log-in for all training, document management, surveys, and data collection tools. This consistent experience ensures sites become invested in using the platform and achieve long-term operational efficiencies.

**ADAPTABILITY**

Within this common environment, Alnylam is able to accommodate the unique needs of each study and create custom tools to manage challenging protocols.

**CUSTOMIZATION**

IQVIA Technologies professional services experts work with Alnylam's clinical teams to develop highly customized tools for specific protocol challenges, ensuring even the most complex studies can be accommodated within the platform.

## Result

IQVIA's Investigator Site Portal has been an enabling technology for Alnylam's patient-first approach, which has proven to be a winning strategy for the company.

Since adopting the technology for its first study in 2014, Alnylam has created an entirely new class of medications known as RNAi therapeutics. Its third product — LUMO™ (lumasiran) — received approval in November 2020 in both the US and Europe to treat Primary Hyperoxaluria Type 1 (PH1). It is the first RNAi therapeutic approved in the U.S. for use in both children and adults and the company's third RNAi Medicine to receive FDA approval in less than three years.

Alnylam's intense focus on patients of every age group with rare and even ultra-rare diseases, along with the dedicated investigators and site staff that treat these brave individuals, has taken lumasiran from identification of compound to regulatory approval in just six years.

**IQVIA Technologies and the Investigator Site Portal have been with Alnylam every step of the way.**

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**IQVIA Investigator Site Portal at Alnylam: Immediate Benefits. Long-term Success.**

**CRA burden removed**

**Patient safety maximized**

**Protocol deviations reduced**

**Recruitment efforts optimized**

## About Alnylam

Alnylam (Nasdaq: ALNY) is leading the translation of RNA interference (RNAi) into a whole new class of innovative medicines with the potential to transform the lives of people afflicted with rare genetic, cardiometabolic, hepatic infectious, and central nervous system (CNS)/ ocular diseases. Based on Nobel Prizewinning science, RNAi therapeutics represent a powerful, clinically validated approach for the treatment of a wide range of severe and debilitating diseases.

Founded in 2002, Alnylam is delivering on a bold vision to turn scientific possibility into reality, with a robust RNAi therapeutics platform. Alnylam's commercial RNAi therapeutic products are ONPATTRO® (patisiran), GIVLAARI® (givosiran), and OXLUMO™ (lumasiran), as well as Leqvio® (inclisiran), which is being developed and commercialized by Alnylam's partner Novartis. Alnylam has a deep pipeline of investigational medicines, including six product candidates that are in late-stage development.

Alnylam is executing on its "Alnylam P5x25" strategy to deliver transformative medicines in both rare and common diseases benefiting patients around the world through sustainable innovation and exceptional financial performance, resulting in a leading biotech profile. Alnylam is headquartered in Cambridge, MA. For more information about our people, science and pipeline, please visit www.alnylam.com and engage with us on Twitter at @Alnylam or on LinkedIn.

**Orchestrate Outcomes**

## About the IQVIA Investigator Site Portal

The IQVIA Investigator Site Portal is a collaborative site-facing technology, widely adopted across the industry for end-to-end study conduct. Delivered in either a functional service provider or softwareas-a-service model, the solution consists of modules and tools that make life easier for sites and help the industry complete more trials successfully. Learn more at iqvia.com/InvestigatorSitePortal.

## About IQVIA Technologies

IQVIA Technologies develops purpose-built solutions to enable life science organizations to orchestrate better outcomes across the entire product lifecycle. For more information about Orchestrated Clinical Trials, visit www.iqvia.com/OCT.

IQVIA TECHNOLOGIES

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