CDD VAULT®
Complexity Simplified

Customer Case Study

"Get a digital platform in place as soon as possible, don't wait for data to start becoming a problem." Anna Orlova, PhD, Co-founder & CEO, RIANA Therapeutics

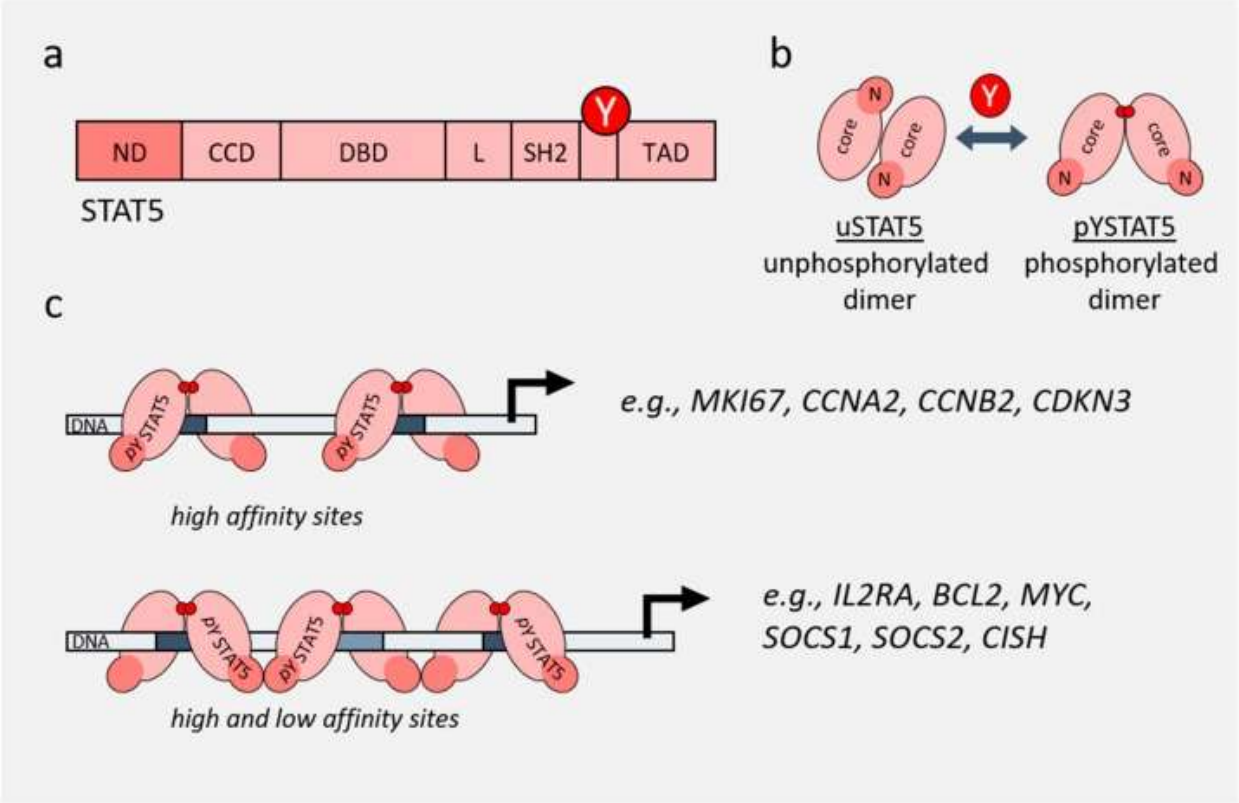
RIANA THERAPEUTICS

RIANA Therapeutics Redefines the 'Undruggable': Translating Academic Discovery into Targeted Cancer Therapies

Drug discovery company RIANA Therapeutics was spun out of the University of Veterinary Medicine, Vienna (Vetmeduni) in 2023 to develop small molecule anticancer therapeutics that target protein-protein interactions (PPIs) in what has traditionally been considered the largely undruggable molecular space of transcription factors.

RIANA's R&D aims to expand on and commercialize research pioneered in the lab of co-founder Professor Richard Moriggl, PhD, who has been working in the field of STAT transcription factors for more than 20 years. The company's initial focus is on developing treatments for blood cancers - primarily acute myeloid leukemia - that target oligomerization of the transcription factor STAT5, explains RIANA co-founder and CEO, Anna Orlova, PhD. This molecular mechanism has been implicated in cancer transformation in multiple types of blood and other cancers, and in resistance to existing cancer therapies.

In parallel to continued work on the STAT5 program, RIANA's ongoing research has indicated potential strategies for therapeutically targeting other oncogenes. The pipeline of potential compounds and disease indications will be "led by the science and discoveries," Orlova noted.



Functions of STAT5 Credit: https://pubmed.ncbi.nlm.nih.gov/31817042/

Phenotypic Screening at the Core of RIANA's Discovery Platform

Importantly, Orlova's academic research at the university and continuation of that research by RIANA is supported by CDD Vault, which Orlova adopted prior to setting up RIANA as a unifying data repository and management infrastructure for the evolving program's chemical structures and screening data. CDD Vault's single user interface, broad suite of functionalities and visualization tools, and secure electronic laboratory notebook (ELN), offer intuitive, easily accessible tools through which RIANA biologists can handle diverse data sets.

Orlova has been studying the cancer-related activity of STAT5 and related transcription factors alongside Moriggl for nearly a decade, initially as part of her PhD program in the Moriggl lab. Retaining laboratory and office space at the Vetmeduni, RIANA has an exclusive license to proprietary phenotypic cell screening technology developed at the university that underpins the drug discovery programs. Results from a diversity compound library screen on the assay platform has generated a series of compound hits exhibiting promising potency for inhibiting STAT5 oligomerization.

The ultimate aim for RIANA will be to license out its lead anticancer compounds for commercialization. "What makes RIANA different in this anticancer drug discovery space is that we're focusing on targeting transcription factors, which are traditionally considered 'undruggable'" Orlova stated. "We're trying to prove this is wrong."

Strategic Digital Foundations

It was in 2020 that Orlova - realizing the value of installing a digital solution even for early-stage R&D - brought CDD Vault into the research lab to manage the program's chemistry data.

Joe Lewis, who mentored Orlova during her time at EMBL Heidelberg and is now at Anavo Therapeutics, originally recommended CDD Vault for managing chemistry data, even when the program was in its earliest academic stages.

"Joe was a big fan", Anna adds. "... even though I was only working with 10 or so compounds at the time.".

Integrating a digital data management platform into the academic lab environment – something that Orlova advocates all labs in similar life sciences research environments should consider – supported seamless corralling and spin-out of the research IP into RIANA.

When, in 2023, Orlova secured an initial tranche of seed funding to set up RIANA, the CDD Vault license was easily transferred from Vetmeduni to the new startup, along with the screening IP and relevant scientific data generated through the university-based research. "When I first started my research as a PhD student with Professor Moriggl, the aim was always to translate that research into the clinic, with a view to developing anticancer treatments," Orlova explains. "The translational grant enabled us to establish RIANA and work towards commercialization."

RIANA team Credit: Tobias Suske

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-Anna Orlova, PhD, Co-founder & CEO, RIANA Therapeutics

Scalable Infrastructure for Research and Collaboration

The new company started operations with an established, flexible digital infrastructure for managing data, which offered security and a complete audit trail, Orlova states. Importantly, CDD Vault has been developed to support fast adoption. A short walk-up time means users can quickly learn how to navigate and if necessary, fine tune the digital tools, minimizing training time and associated costs. For the Morrigl lab and RIANA this was especially valuable as the research was progressing towards translational potential.

"Initially, CDD Vault served as a secure digital repository for storing data relating to our chemical structures. It was somewhere to put the data." Orlova acknowledges.

"As a biologist, not a chemist, I'd never used anything like the Vault before, and so initially I

may have been a bit reluctant." However, she continues, the Vault soon became invaluable for managing, comparing and interrogating the structural and other chemistry-related data with which biologists may not be so familiar.

Employing the Vault as the lab's primary data repository means that researchers are freed from reliance on spreadsheets or paper-based for managing data, Orlova comments. "The Vault makes it easy to find, access and view or check information almost instantly." Adoption of the Vault early also meant that there was no period of disruptive transition – and so minimal risk of migration errors – associated with transferring historical data from paper and spreadsheets into the digital domain.

Efficient Protocol Management and Regulatory Readiness

Since establishing RIANA, Orlova and the team set up Vault ELN for managing experimental protocols, and for recording and reporting on biological assays and in-house research. "The Vault ELN has been our day-to-day platform for managing our experimental protocols and results," Orlova says. "I absolutely hated those paper protocol books that can be hard to read and make sense of. Recording in paper notebooks means that information can be hard to find, may be incomplete, or get lost completely."

Recording data in the ELN also provides a complete audit trail, saves time and aids standardization, Orlova points out. For our research, it's really important to have full, complete data recorded, reported, easily accessible and fully traceable." Storing all experimental protocols and data in the ELN provides transparency, so that the team can all see who is working on what, and this helps the scientists work more efficiently and collaboratively and provide each other with help when needed.

"We're constantly increasing our suite of standardized protocols, stored in the ELN - I think we're up to 60 or more now, from just four a couple of years ago – and the system makes it easy for us to create templates so that the researchers don't have to type everything out every time they carry out a common procedure. They can just paste in the data that doesn't change, from a previous workflow, and type in the fields that are the variables."

> ***"The Vault allows us to look at the data within and between different experimental protocols, and potentially identify where things may have gone wrong," she continues. "And when I need to access data from two years ago, I can find it in the Vault within minutes."***
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Built for Discovery and Data-Driven by Design

RIANA operates a pretty straightforward drug discovery and development model, Orlova notes. "It's a traditional model founded on high throughput screening, validation, and optimization to create lead compounds that can then be progressed into preclinical testing. And

it's astonishing how fast the amount of experimental data we generate increases and expands. "We can configure the Vault to suit our needs," she adds, indicating that without the Vault architecture as a central repository, the expanding datasets would have become difficult to store and manage intuitively.

The combination of features available to Vault users makes the routine tasks of data management faster and simpler, Orlova points out. "The Vault offers a safe, user-friendly structural platform with visualization tools that make it easy to relate and compare data, and to troubleshoot. Everyone using the Vault has access to the same data, in real time, and we can organize and share data in the best way for communicating both within the team, and to our partners and to potential investors. It's super practical."

The Vault in addition allows the scientists to review research progress and ensure that there are no gaps– an experiment that may be lacking, for example, Orlova comments. "We can make sure that there's no unnecessary replication of work that's already been carried out." Similarly, data can be compared to identify unexpected or anomalous results that may point to the need to repeat work. "The Vault allows us to look at the data within and between different experimental protocols, and potentially identify where things may have gone wrong," she continues. "And when I need to access data from two years ago, I can find it in the Vault within minutes."

Four of the five RIANA team are biologists, and while the company has lab space for carrying out the biology in house, the chemistry is outsourced. "Screening data coming in can be uploaded directly into the Vault with no loss of content or detail, and when new compound structures arrive I can cluster the data in the Vault in the most appropriate way – by scaffold, for example – and then export all the relevant data to our chemistry partners. It takes perhaps 10 minutes for me to download the data, add some color coding, and then it's good to go." The visualization tools make it quick to carry out on the spot comparisons, check correlations, and create fast graphical representations for use in house, she continues.

The Vault gives confidence in data quality and completeness, Orlova maintains, and this is important when considering the prospective future need to meet regulatory compliance and audit imperatives as development moves towards IND applications. It's never too early to have fully auditable and transparent data management and manipulation trails, even well in advance of starting preclinical in vivo studies, Orlova suggests. Data security is native to the Vault architecture, with access and permissions set for each user.

Importantly, use of the Vault on a daily basis is straightforward, Orlova adds, and little ongoing support is needed. "The CDD team provides updates to the platform as released, and they are there should we need support or have queries. We also meet and catch up at events during the year." While minimal support may be needed for the RIANA team, CDD Vault does include comprehensive services such as implementation support, user training, onboarding assistance, and regular maintenance and upgrades, all provided at no extra or hidden cost. These services are built into the offering, ensuring customers are fully supported throughout their journey, from initial rollout to day-to-day use.

Advice for Future Founders

Asked what she would advise scientists or labs looking to create a start up from academia, and the short answer from Orlova is, “Get a digital platform in place as soon as possible, don’t wait for data to start becoming a problem.” Start thinking about potential data management bottlenecks well in advance, she stresses. “Never underestimate how fast the volume and depth of data can grow and potentially become unmanageable for traditional paper/spreadsheet-based tools. And overcome any ‘fear’ of utilizing unfamiliar tools by just taking things one step at a time.”

Benefits of Using CDD Vault for RIANA Therapeutics

Quick Implementation: Easy to adopt with minimal training- ideal for fast-paced early-stage research.

Seamless IP Spin-Off: Enable structured and secure migration of data assets and experimental workflows from academia to startup settings, preserving integrity and reproducibility.

Unified, Cross-Functional Interface: A broad toolset within a single, intuitive interface, designed to be accessible to both biologists and chemists, enabling seamless collaboration across disciplines. This includes support for biologist who increasingly work with chemistry data.

About Collaborative Drug Discovery

Collaborative Drug Discovery provides a modern approach to drug discovery informatics trusted globally by thousands of leading researchers. Our CDD Vault is a hosted informatics platform that securely manages both private and external biological and chemical data. It provides core functionality including chemical registration, structure-activity relationship, inventory, visualization, and electronic lab notebook capabilities. For more information, visit us at www.collaborativedrug.com.