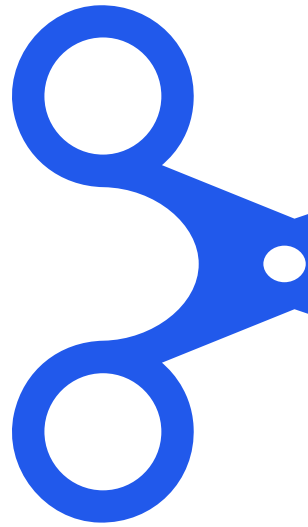
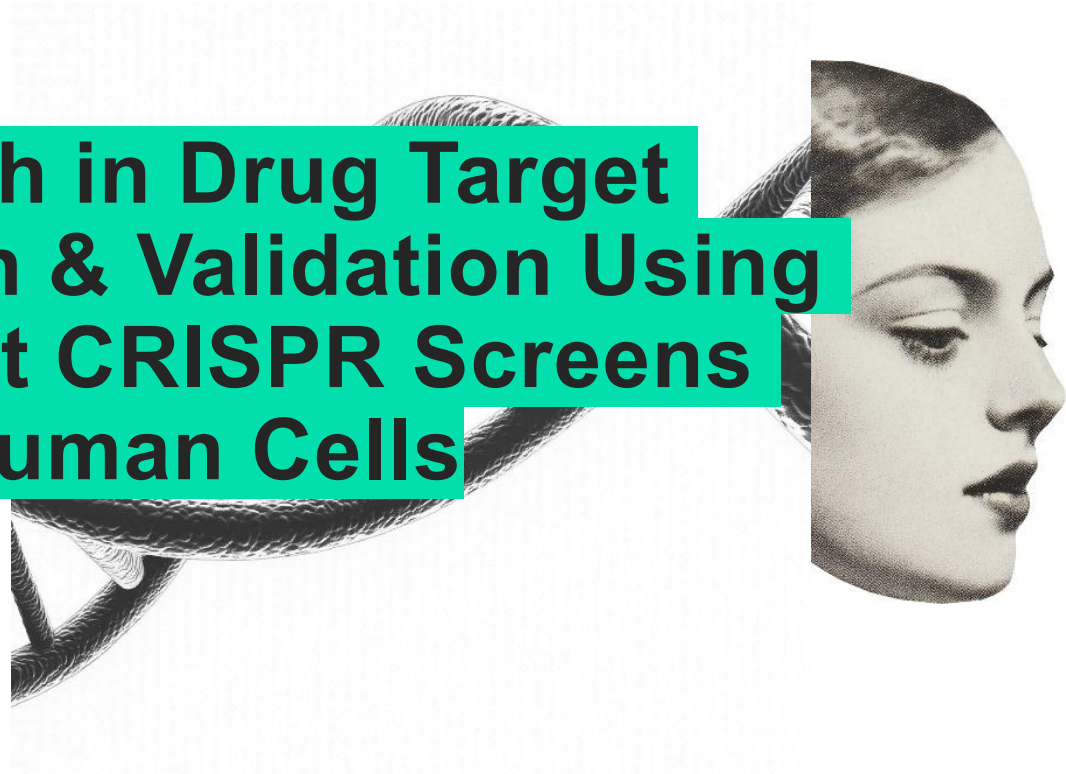


myllia.



Q1 2026

**Breakthrough in Drug Target
Identification & Validation Using
High-Content CRISPR Screens
in Primary Human Cells**



About Myllia | Our Fundamentals



- Myllia was founded in 2018 by Thomas Moser (CEO), Tilmann Buerckstuemmer (CSO) and Christoph Bock (Scientific Advisor and Principal Investigator at CeMM)
- Myllia's technology platform applies next-generation CRISPR screens for drug target discovery in primary human cells
- Myllia works with biotech and pharma customers from EU, UK, US and JP who access Myllia's platform for target identification and target validation on a fee-for-service basis
- Myllia's teams have conducted 100+ CRISPR screens across 30+ projects
- 25+ FTEs operating in a 11,000 square feet facility in Vienna, Austria



Guided by experience, driven by discovery



Dr. Thomas Moser | CEO

- 10+ years as Managing Partner at Pontis Capital
- Vice President Products and Head of Corporate Strategy at Horizon
- Long-standing history advising start-up companies



Dr. Christoph Bock | Advisor

- Principal Investigator at CeMM
- Research: Single-cell RNA sequencing, high-throughput technologies and bioinformatics



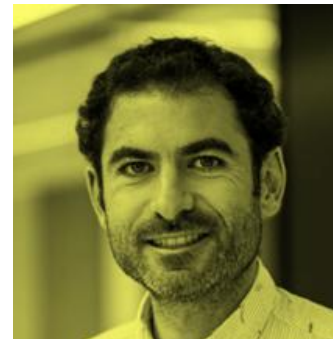
Dr. Tilmann Bürckstümmer | CSO

- Head of Innovation with global responsibility at Horizon Discovery
- Serial entrepreneur
- Creative thinker with 20+ publications and 10+ patent applications



Dr. Luke Gilbert | Advisor

- Professor at UCSF and the Arc Institute
- Developed CRISPRa and CRISPRi
- Research: Single-cell CRISPR screens in oncology
- Co-founder of Chroma Medicine



Dr. Rodrigo Santos | Advisor

- Horizon Discovery, bit.bio and Mogrify
- Entrepreneur in Residence at Novo Holdings
- CTO at clock.bio



GSK has partnered with Myllia to leverage their single-cell CRISPR screening platform for identifying novel drug targets across the following therapeutic areas:

1. CRISPR screens in primary human T cells to uncover novel targets in autoimmune disease and immuno-oncology
2. CRISPR screens to identify novel targets in solid tumors
3. CRISPRi (CRISPR interference) screens to modulate target gene expression



“We have worked with Myllia on a target discovery project. Their single-cell CRISPR screening platform is cutting-edge and delivered both known and intriguing novel hits. We will definitely consider Myllia for future projects.”

Oliver Rausch,
Chief Scientific Officer



Cell Reports

Article

Genetic dependencies associated with transcription factor activities in human cancer cell lines

Venu Thatikonda,^{1,4,*} Verena Supper,¹ Johannes Wachter,¹ Onur Kaya,¹ Anju Kombara,¹ Ceren Bilgili,¹ Madhwesh C. Ravichandran,¹ Jesse J. Lipp,¹ Rahul Sharma,³ Lukas Badertscher,³ Andrew S. Boghossian,² Matthew G. Rees,² Melissa M. Ronan,² Jennifer A. Roth,² Sarah Grosche,¹ Ralph A. Neumüller,¹ Barbara Mair,¹ Federico Mauri,¹ and Alexandra Popa^{1,5,*}

¹Boehringer Ingelheim RCV GmbH & Co KG, Doktor-Boehringer-Gasse 5-11, Vienna 1120, Austria

²Broad Institute of MIT and Harvard, Cambridge, MA 02142, USA

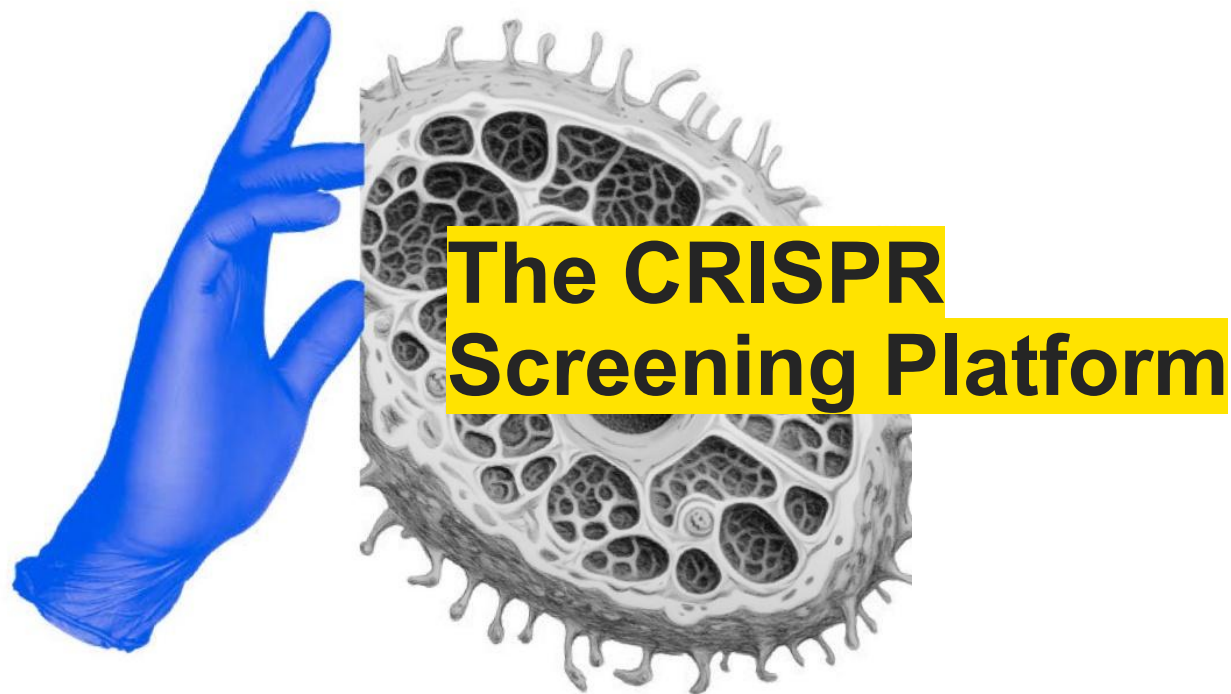
³Myllia Biotechnology GmbH, Am Kanal 27, Vienna 1110, Austria

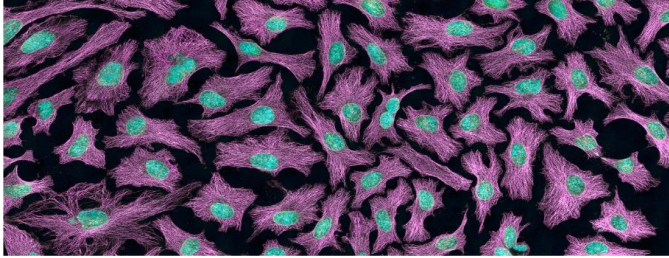
⁴Present address: Exscientia GmbH, Siemensstraße 89/OG3, Vienna 1210, Austria

⁵Lead contact

*Correspondence: vthatikonda@exscientia.co.uk (V.T.), alexandra.popa@boehringer-ingelheim.com (A.P.)

<https://doi.org/10.1016/j.celrep.2024.114175>





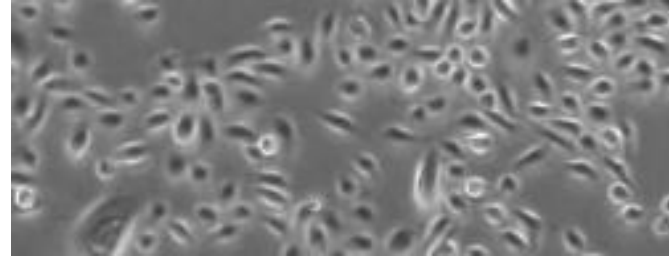
Cancer Cell Lines

Why are they still so popular?

- Easy to culture
- Easy to propagate
- Homogenous
- Easy to get CRISPR to work

What are the limitations?

- Abnormal karyotype
- Abnormal growth
- Limited physiology relevance
- Drug targets do not translate



Primary Human Cells

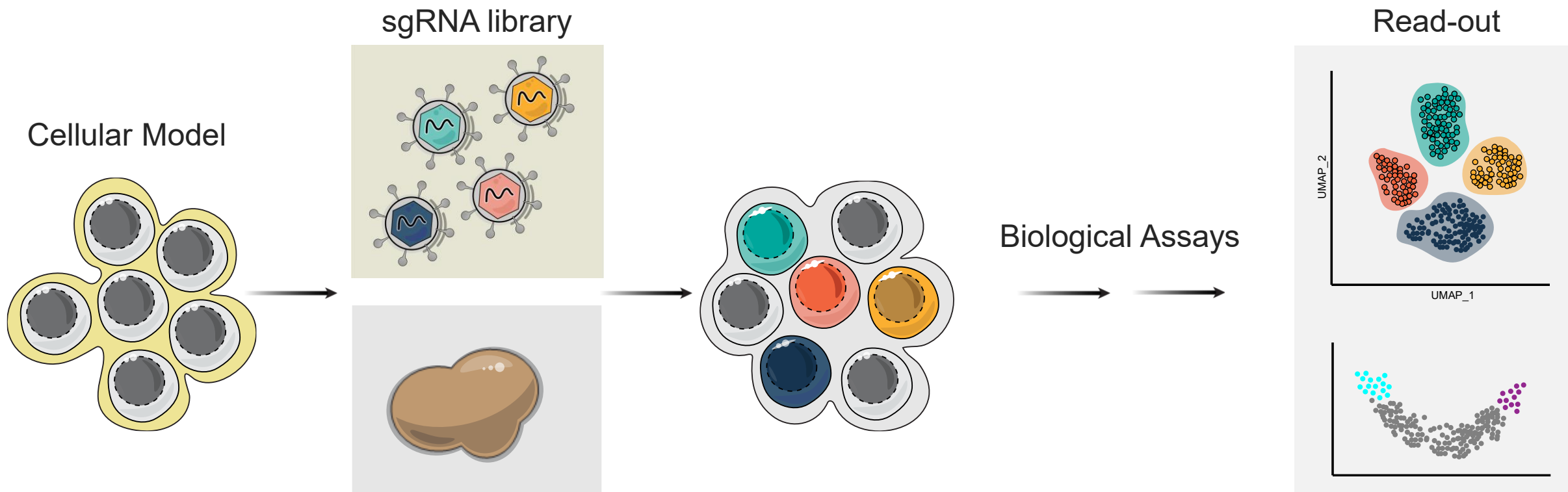
Why is nobody (else) using them?

- Difficult to source and culture
- Donor-to-donor variation
- Heterogeneity
- Difficult to get CRISPR to work

What are the key benefits?

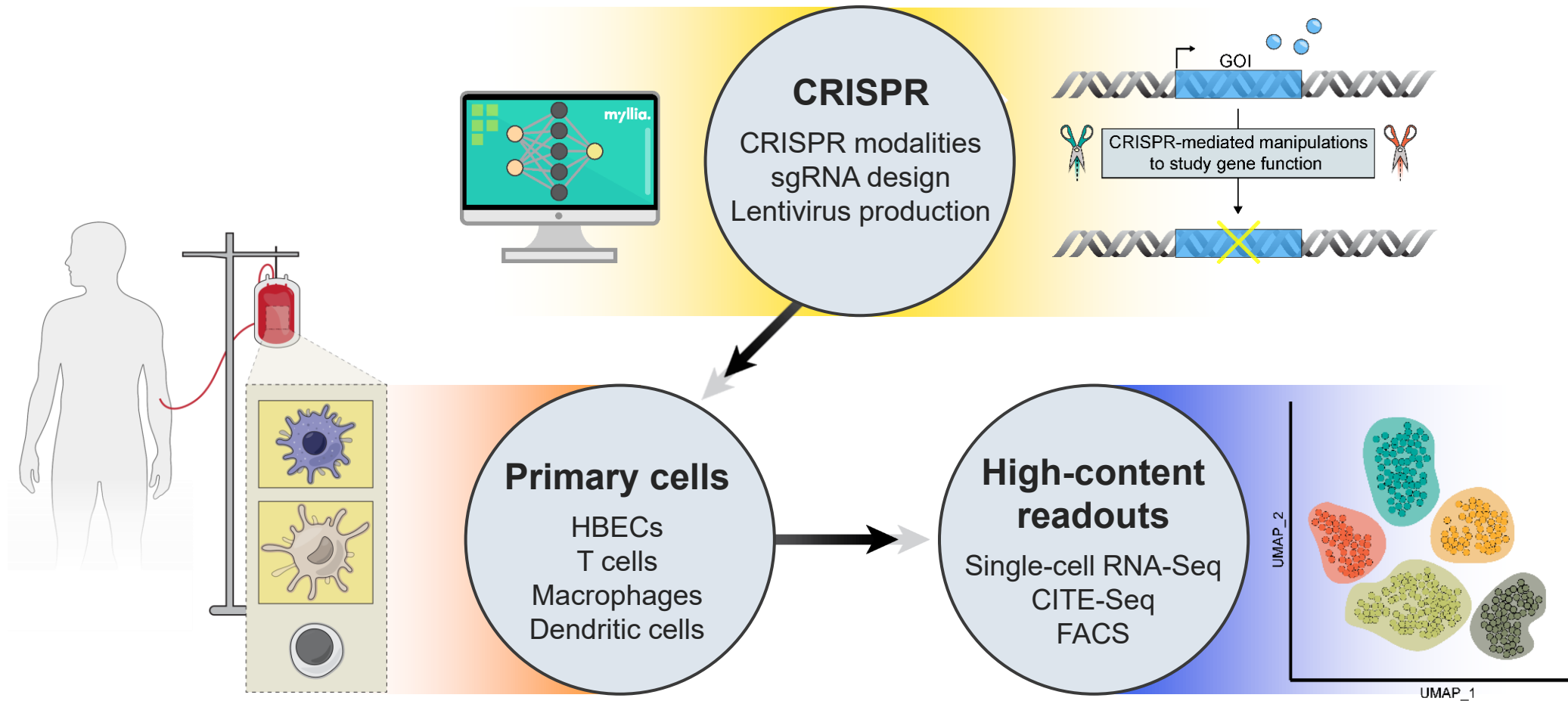
- Normal karyotype
- Higher physiological relevance
- Healthy or diseased
- High predictive value for target identification and validation

CRISPR screens – The ideal approach for target identification and target validation



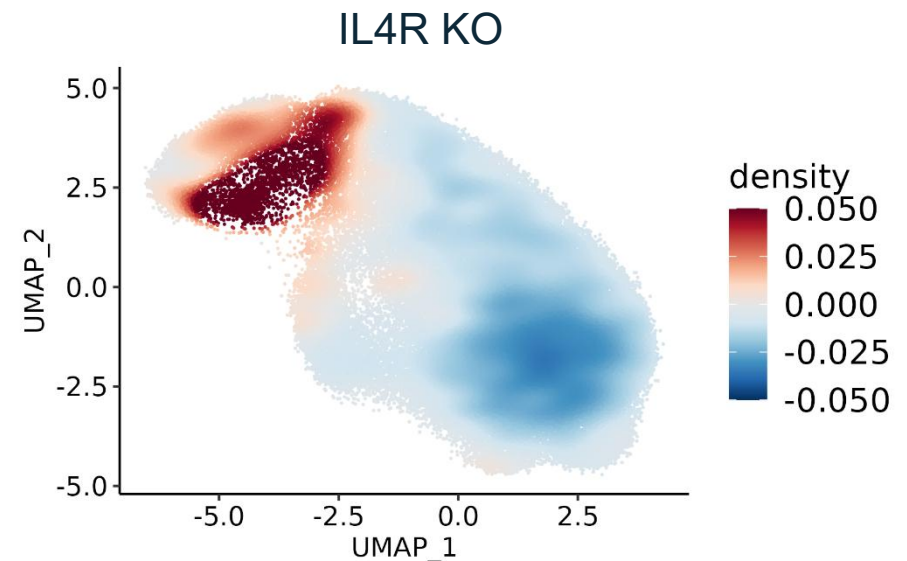
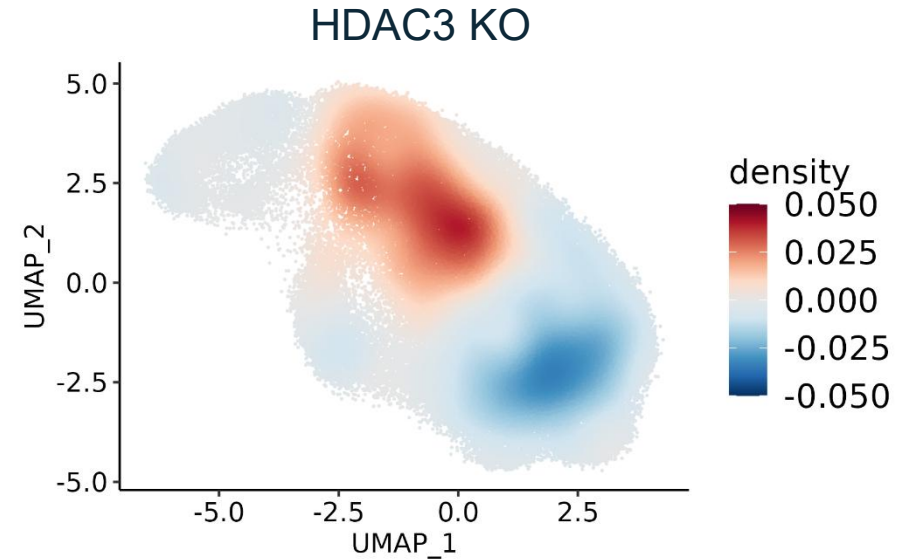
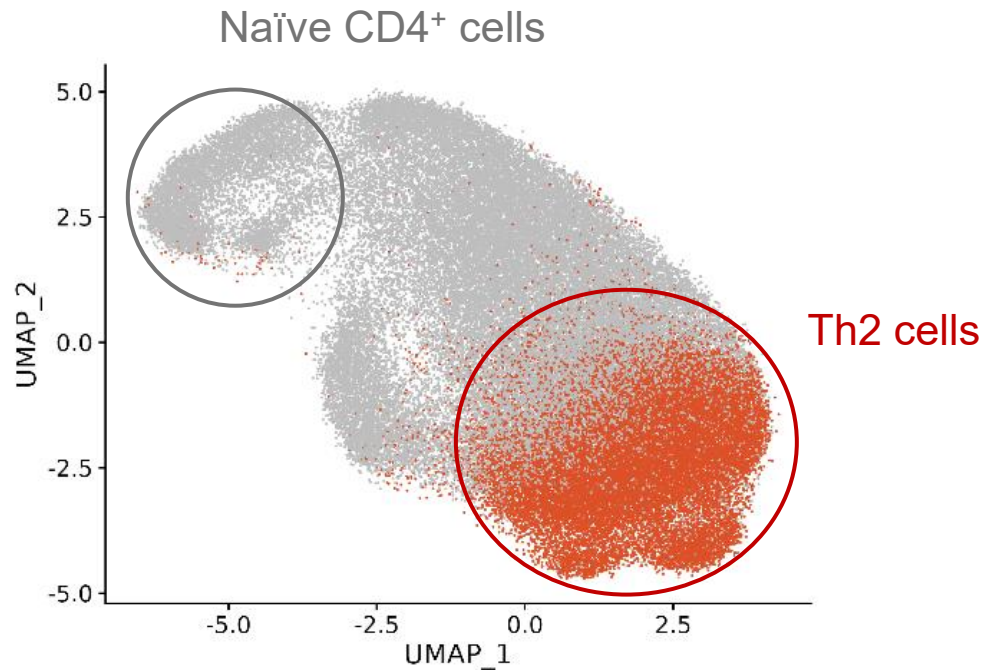
- Choose a cellular model
- Perturb cells with CRISPR/Cas9 and guide RNA library
- Perform a biological assay
- Link the screen to a phenotypic read-out

Myllia's Technology Platform: Unbiased drug target discovery in primary human cells



Single-cell CRISPR screens in primary human T cells

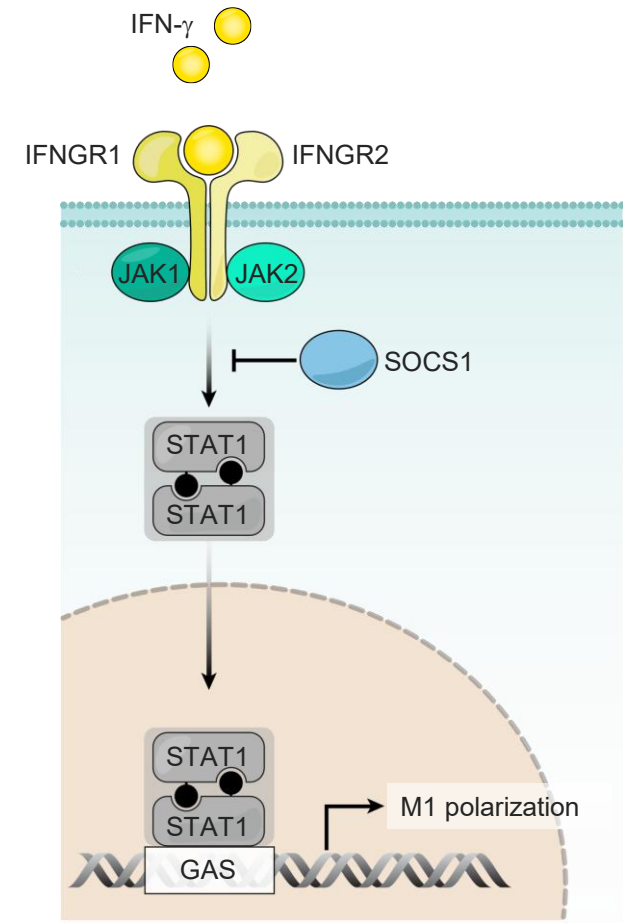
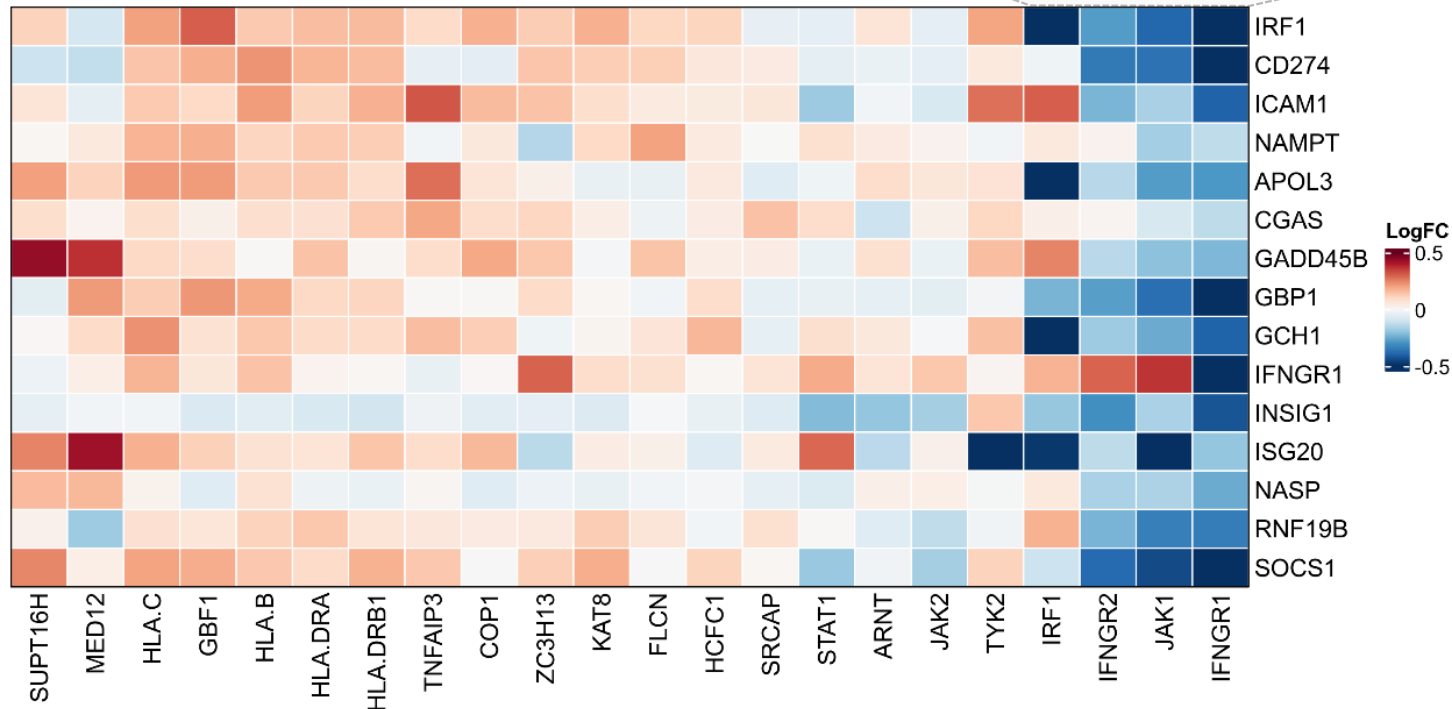
- Obtain primary human CD4⁺ T cells from human blood
- Introduce Cas9 and sgRNA library in pooled fashion
- Polarize towards the desired lineage (Th2)
- Record cell state by single-cell RNA seq (scRNA-Seq) and assess gene KO phenotypes



Dissecting gene function in primary human macrophages

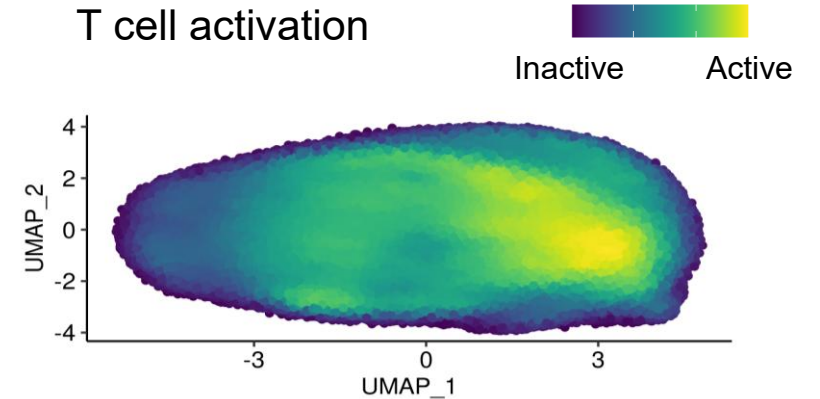
- Obtain primary human monocytes from blood and differentiate into M0 macrophages
- Introduce Cas9 and pooled sgRNA library
- Polarize towards the desired lineage (M0 to M1)
- Record cell state by single-cell RNA-Seq and assess gene KO phenotypes

Gene knockdowns affecting M1 macrophage polarization

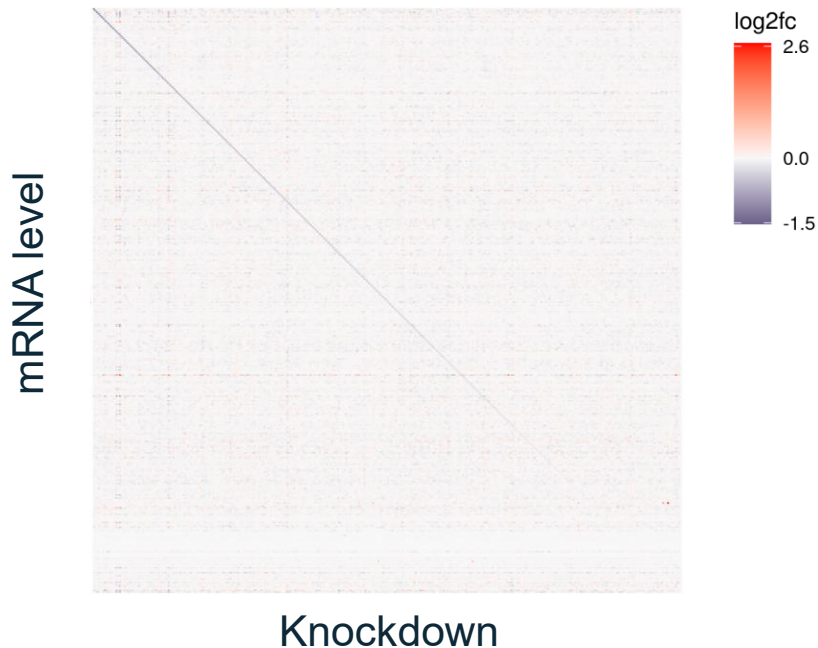


A genome-wide compressed CRISPRi CROP-Seq screen

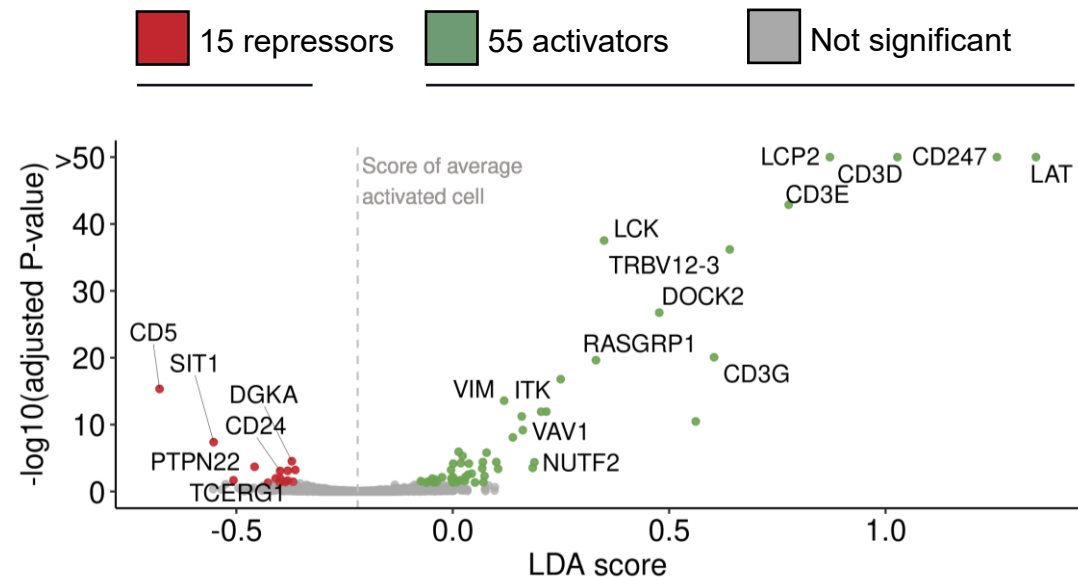
- Perturb Jurkat cells with dCas9-KRAB and genome-wide sgRNA library targeting >19,000 human genes
- Activate cells
- Perform single-cell RNA sequencing in >1 million cells to record T cell activation state



CRISPRi knockdown

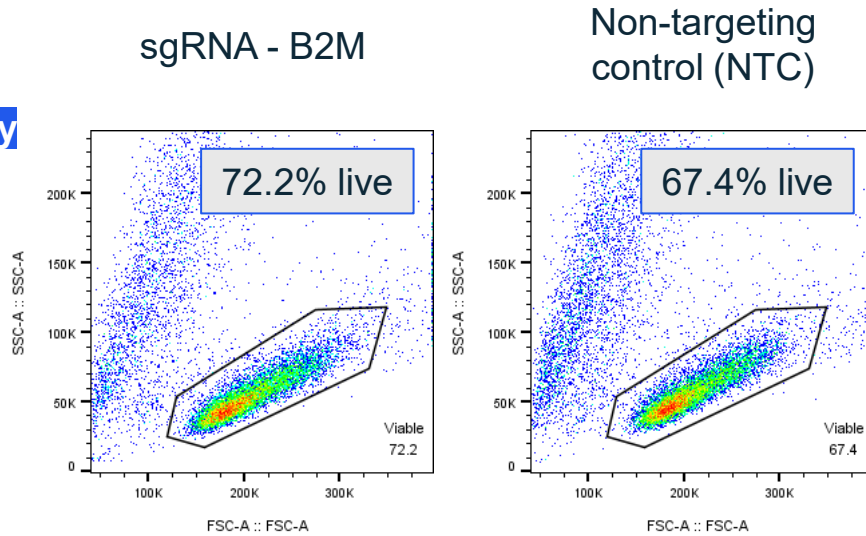


CRISPR perturbation outcomes

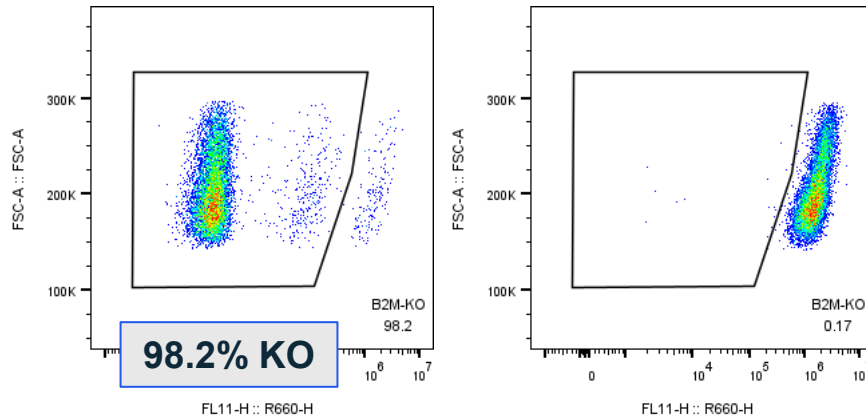


CRISPR/RNP-based arrayed validation of gene targets

Viability

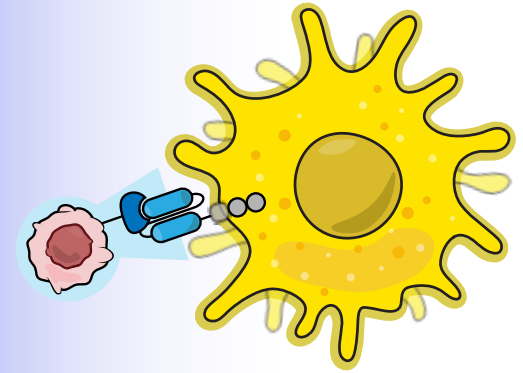


B2M



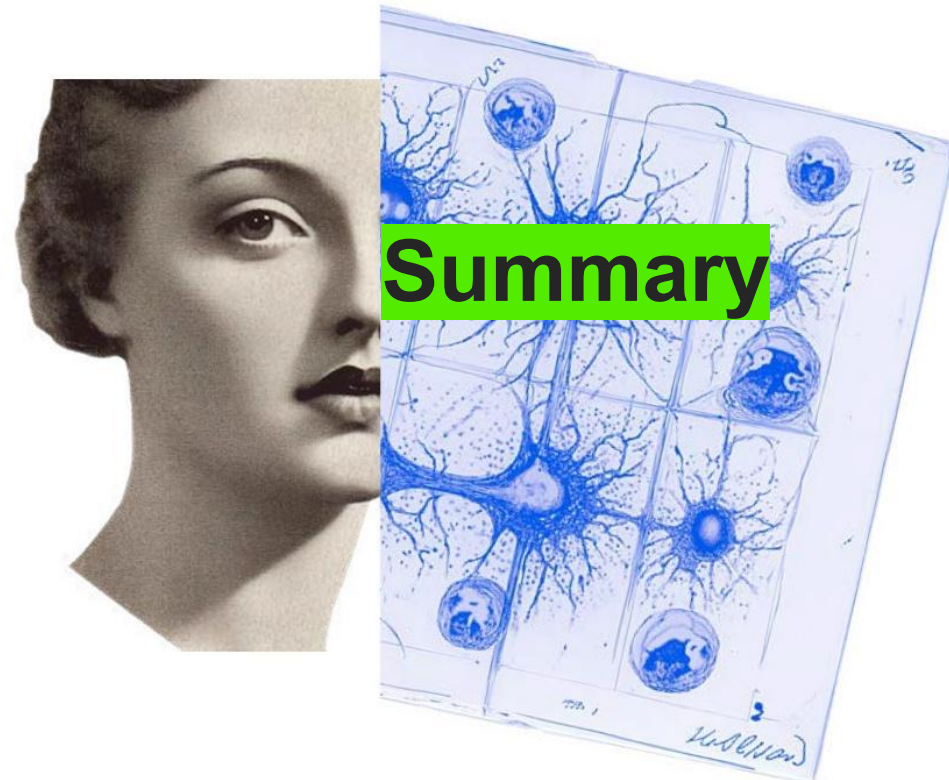
B2M levels in primary human T cells (APC)

Functional validation of novel gene targets



Phenotypic readouts for functional validation

- Viability and proliferation assays
- Bulk RNA-Seq for single gene perturbations
- ELISA for cytokine and/or chemokine secretion
- Flow cytometry for intracellular and/or surface markers
- Imaging of cellular phenotypes: Morphology and signaling
- Co-culture assays for cell-cell-interactions



Fuel your next drug discovery programs with the most potent targets

myllia.



Unbiased Discovery

CRISPR screens deliver the best targets in an unbiased fashion



Primary Human Cells

Target identification happens in the only cells that matter – primary cells from human donors



High Translatability

Targets identified in primary cells *in vitro* have a high chance of translating *in vivo*

How we do business

Each client and project is unique. We shape the scope to match your specific needs.

Tailor-made

Science can be unpredictable. We adjust your project on the flight whenever needed.

Flexible

We work on a simple fee-for-service basis, and the data we generate belong entirely to you.

No strings attached

Please get in touch to discuss your next project

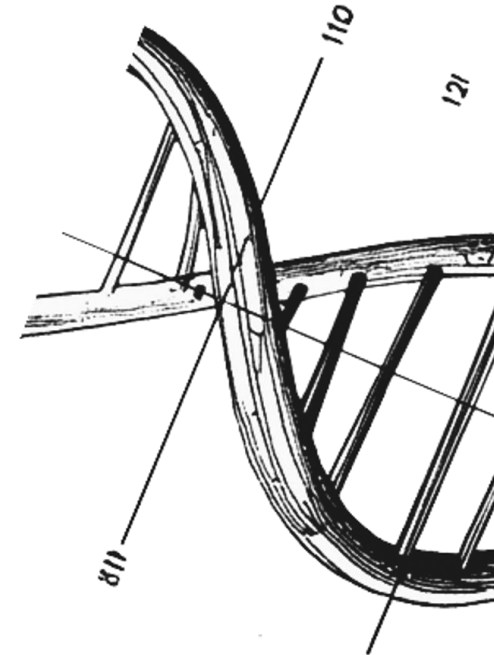
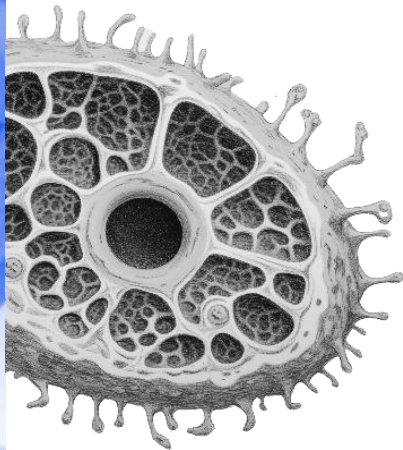
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