

# Myllia Company Presentation

Austrian Life Science Day

Thomas Moser, PhD | CEO

May 2023

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## Myllia Biotechnology - Executive Summary

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- Myllia Biotechnology has been **founded in Feb 2018** in Vienna, Austria by experienced serial entrepreneurs
- Myllia capitalizes on one of the most important trends in modern life sciences. The **single-cell analysis** revolution raises biology to the next level, transforming it into a data-intensive industry with extremely high throughput.
- Myllia combines advanced **single-cell transcriptomics** read-out with challenging, **physiologically relevant** models like primary T-cells or iPSCs
- One of the **largest teams** worldwide focussing exclusively on next-generation single-cell CRISPR screens
- **Fully integrated workflow** from design and implementation of the screen to full bioinformatics analysis and visualization, all performed in-house.
- Conclusion of several **large partnerships with big-pharma companies** in **US, UK and JP**, typically in the area of target discovery and MoA analysis
- Business model focussed on **revenue-generating partnerships** and collaboration deals.

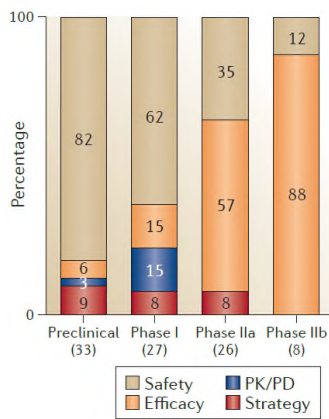




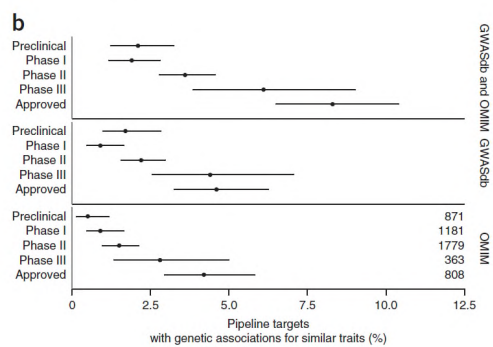
Team of 40+ Scientists  
Dedicated wet-lab and computational biology teams

3

Drugs often fail for lack of efficacy



Drugs hitting genetically validated targets are more successful

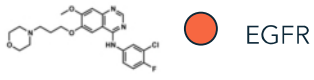
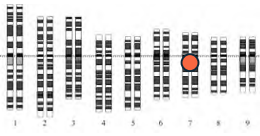


Cook, D., Brown, D., Alexander, R. *et al.* Lessons learned from the fate of AstraZeneca's drug pipeline: a five-dimensional framework. *Nat Rev Drug Discov* **13**, 419-431 (2014). <https://doi.org/10.1038/nrd4309>

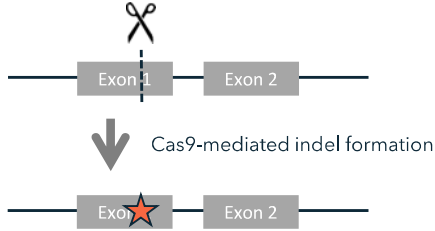
Nelson, M., Tipney, H., Painter, J. *et al.* The support of human genetic evidence for approved drug indications. *Nat Genet* **47**, 856-860 (2015). <https://doi.org/10.1038/ng.3314>

4

Gene products are drug targets



CRISPR/Cas9-mediated gene perturbation



5

CRISPR screen: Unbiased gene perturbation



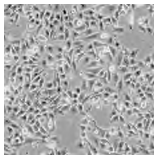
Positive Selection  
Resistance Screens



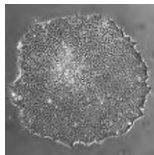
Negative Selection  
Dropout Screens



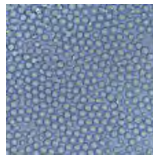
CRISPR screening with single-cell RNA sequencing read-out (CROP-Seq)



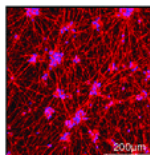
Cancer Cell Line



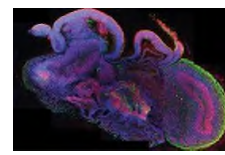
iPSC



Primary T cell

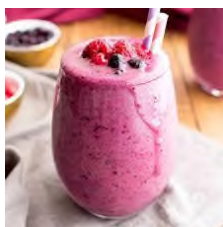


iPSC-derived cell



Organoid

Complexity of the cellular model

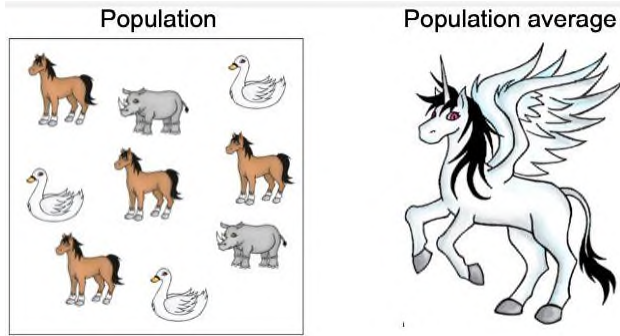


Bulk Screens



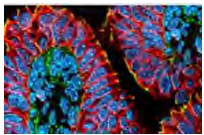
Single-cell Screens

6

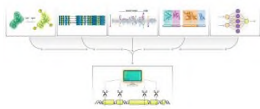


7

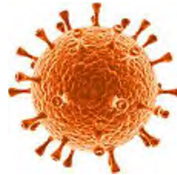
Meshal Ansari and Fabian Theis



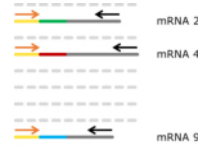
Human cell culture



sgRNA design



Lentiviral Manufacturing

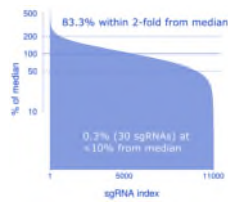


Targeted PCR panels

Cas9 modalities



Pooled library cloning



Single-cell RNA seq



Bioinformatic Analyses



8



Drug target  
identification  
and validation



Mode of action  
analysis



Identification of  
disease-associated  
gene function



Genetic screening  
for phenotypes of  
primary T cells

9



Vienna Biocenter Campus

3 world-class academic institutes (IMP, IMBA, MPL)  
35+ Biotech Companies  
1.500 Biomedical Scientists  
Access to Core Facilities (NGS and FACS)

Headquarter at DOCK27

~600m<sup>2</sup> lab space  
~400m<sup>2</sup> office space  
Newly furnished and newly equipment  
Close to city centre (15min), airport (20min)  
and Vienna Biocenter (10min)

Founding  
Member of



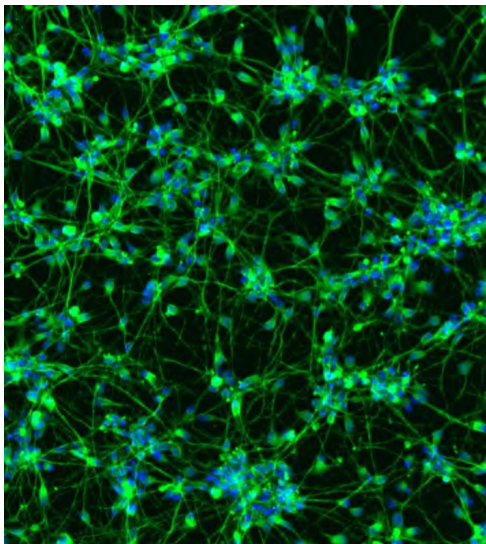
10

GSK has entered into a collaboration with Myllia Biotechnology, applying Myllia's single-cell CRISPR screening platform for drug target discovery in the following areas:

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



11



#### Key Facts

- Joint Venture between Myllia Biotechnology (based in Vienna/ Austria) and bit.bio (based in Cambridge/ UK)
- Focus on **induced pluripotent stem cells** and cells obtained by directed differentiation
- Combines unbiased **CRISPR screens** with the best human cellular models available.

#### Unique selling points

- Best-in-class cellular models
- Custom isogenic disease models
- Unprecedented robustness and consistency
- CRISPR screens with single-cell resolution

bit.bio  
discovery

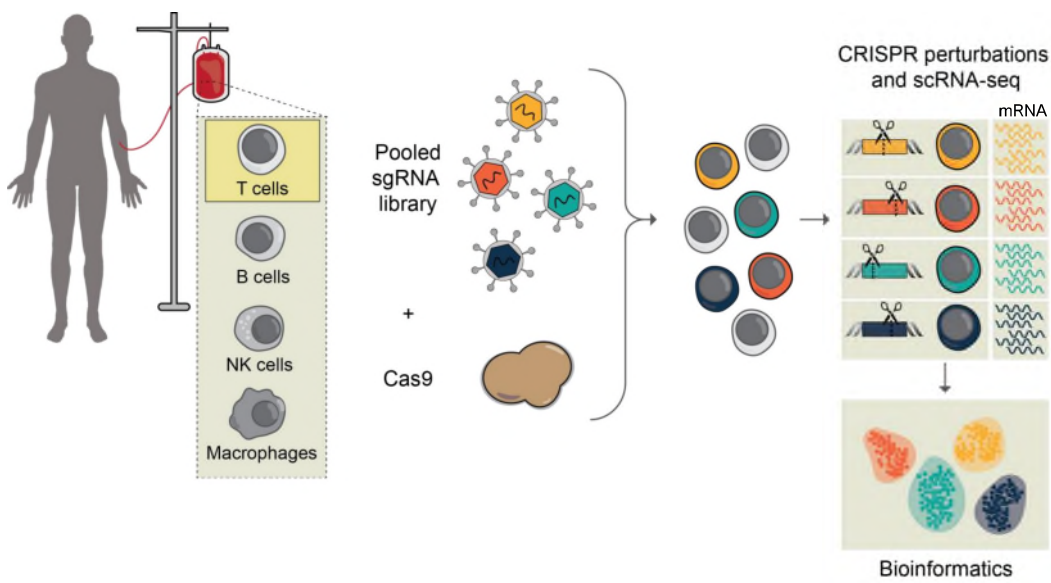
12

# Case Study:

## TCR signalling in primary human T cells

CROP-Seq screens in human primary T cells

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CROP-seq experiment in pan T-cells

- Unactivated cells
- Cells activated with CD3/CD28-Dynabeads for 12 days

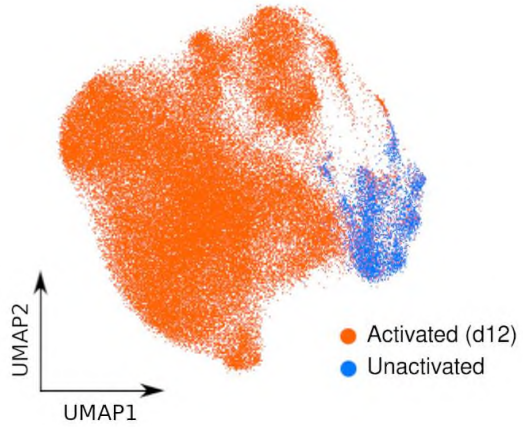
Number of perturbed genes

- 42 genes
- 188 sgRNAs (4 sgRNAs/ gene)

Read-out by targeted single-cell RNA sequencing of 300 mRNAs

- T cell activation markers
- Markers for cell types
- Cell cycle markers
- Immune checkpoint genes

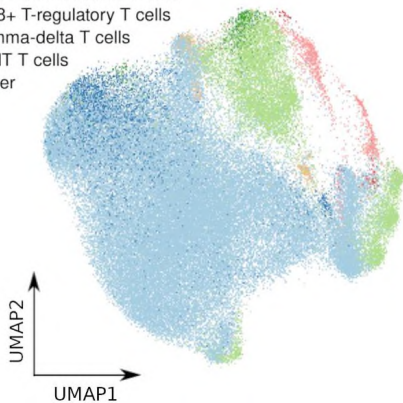
- Unactivated cells (~ 10 %)
- Activated cells (day 12) (~90 %)



Which T cell subsets do we see?

Which effector types do we see?

- CD4+ Conventional T cells
- CD4+ T-regulatory T cells
- CD8+ Conventional T cells
- CD8+ T-regulatory T cells
- gamma-delta T cells
- MAIT T cells
- Other



- Naive T cells
- Central Memory T cells
- Effector Memory T cells
- Effector Memory RA T cells

