
A scientist wearing safety glasses and a lab coat is using a pipette to transfer liquid into small vials. The background is a blurred laboratory setting with various glassware and equipment. A large, semi-transparent purple circle is overlaid on the right side of the image, containing the company's mission statement and logo.

“Our goal is to become a leading biotechnology company, globally recognized for discovering and developing breakthrough small molecule drugs acting on unexplored protein and novel mRNA targets to help patients in need of innovative therapeutic solutions”

molecule

Fate can be altered

April 5, 2023

A background image of a scientist in a white lab coat and safety glasses, using a pipette to transfer liquid into a multi-well plate. The image is overlaid with a semi-transparent blue circle on the right side.

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Warsaw, April 5, 2023

Presenting Team

Marcin Szumowski
Chairman of the Board & CEO



LSI
LIFE SCIENCE INNOVATIONS FUND

Zbigniew Zaślona
Chief Scientific Officer



sitryx

Samson Fung
Chief Medical Officer



argenx



morphosys

Sławomir Broniarek
Chief Financial Officer



opera.pl
OPERA S.A. - Otwarty Fundusz Emerytalny

2022 | Two clinical assets enter Molecure's pipeline

Revision of pipeline priorities

OATD-01: preparations for a Phase II Proof-of-concept (PoC) clinical trial

OATD-02: regulatory approval and start of a Phase I clinical trial in solid tumor patients

Progress in the deubiquitinase (DUB) platform

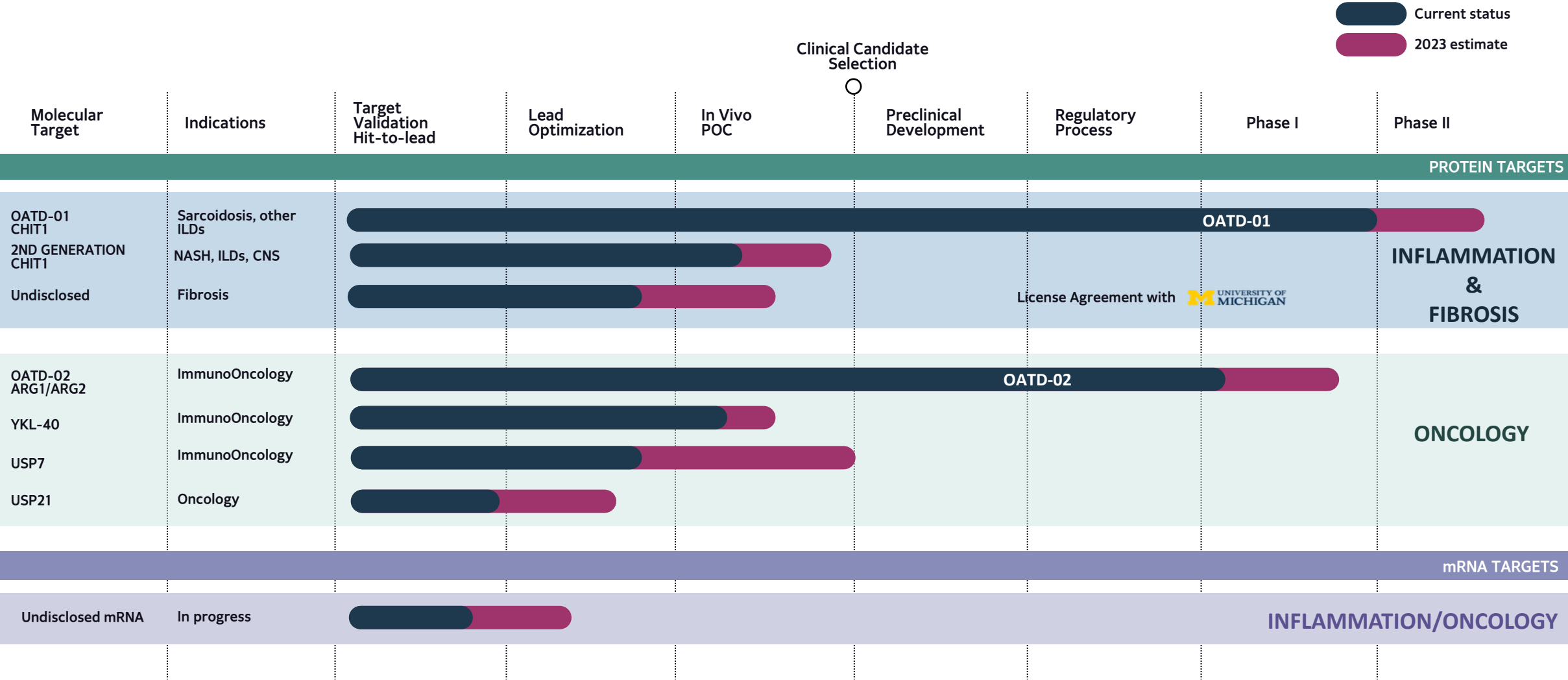
Further development of mRNA platform

Discovery of antifibrotic drugs based on a new target and license from UoM*

Change of the Company's name to Molecure

Team and infrastructure expansion

Balanced pipeline



A scientist wearing safety goggles and a lab coat is using a pipette to transfer liquid into a test tube. In the background, there are various laboratory glassware including a graduated cylinder, an Erlenmeyer flask, and a rack of test tubes. The scene is overlaid with a semi-transparent blue circle.

OATD-01

OATD-01 | on track to enter Phase II

Preparations for Phase II clinical trial in patients with pulmonary sarcoidosis

Ph I studies in human volunteers performed (129 subjects exposed)

Clinical trial plan in collaboration with a group of key opinion leaders and clinicians from the US and Europe*

OATD-01

Grant application for the Phase II clinical trial to ABM** for a total amount of PLN 49m submitted (PLN 24.5m grant funding)

Application for funding of US part of clinical trial to the NIH** submitted (USD 2.2m)

2 articles published*** by Molecure describing the results obtained regarding OATD-01

*including Prof. Marc Judson, Daniel Culver, Marlies Wijisenbeek, Prof. Michael Kreuter, and Prof. Vincent Cottin,

**NIH - the National Institutes of Health, ABM – Agencja Badań Medycznych

***the European Journal of Pharmacology: "Inhibition of CHIT1 as a novel therapeutic approach in idiopathic pulmonary fibrosis" (publication date 15 March) and the Journal of Inflammation Research: "Pharmacological Inhibition of Chitotriosidase (CHIT1) as a Novel Therapeutic Approach for Sarcoidosis" (publication date 29 September).

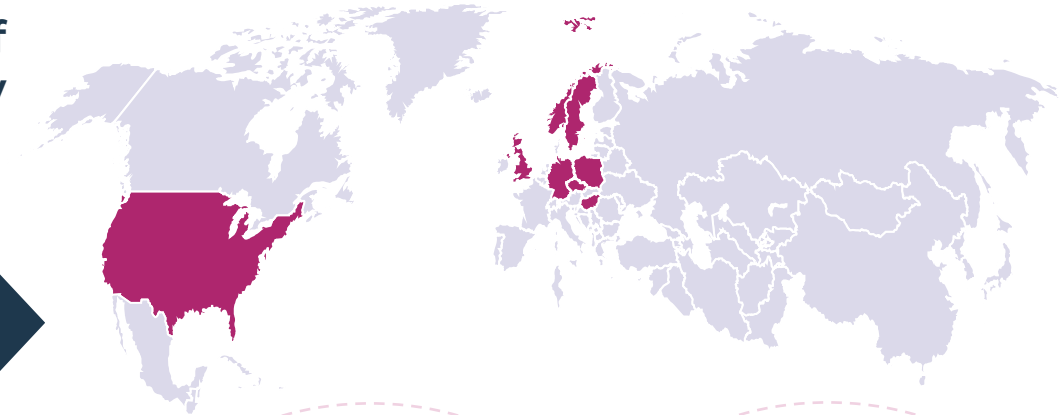
OATD-01 | Phase II clinical trial plan

Double-blind, randomized, placebo-controlled multi-center study to assess the safety and efficacy of an oral inhibitor of CHIT1 (OATD-01) in patients with active pulmonary sarcoidosis



Endpoints

- Between-group difference in granulomatous inflammation (PET-CT imaging)
- Difference in pulmonary function test
- Difference in time to symptom improvement
- Difference in number of patients escaping to corticosteroids
- Quality of life measurement



20 to 30 out-patient sites in the EU and US

~90 male and female patients with active pulmonary sarcoidosis

Each patient treated for 12 weeks

OATD-02



OATD-02 | administered to the first patient



Design: Open-label single-arm dose-escalation monotherapy study (Bayesian design, 2.5-30mg)



Location: 3 sites in Poland: Warsaw, Otwock, Bydgoszcz



Patient population (30-40 patients): Relapsed/refractory advanced and/or metastatic solid tumors

Colorectal cancer, platinum-resistant serous ovarian cancer, pancreatic ductal cancer, renal cell carcinoma

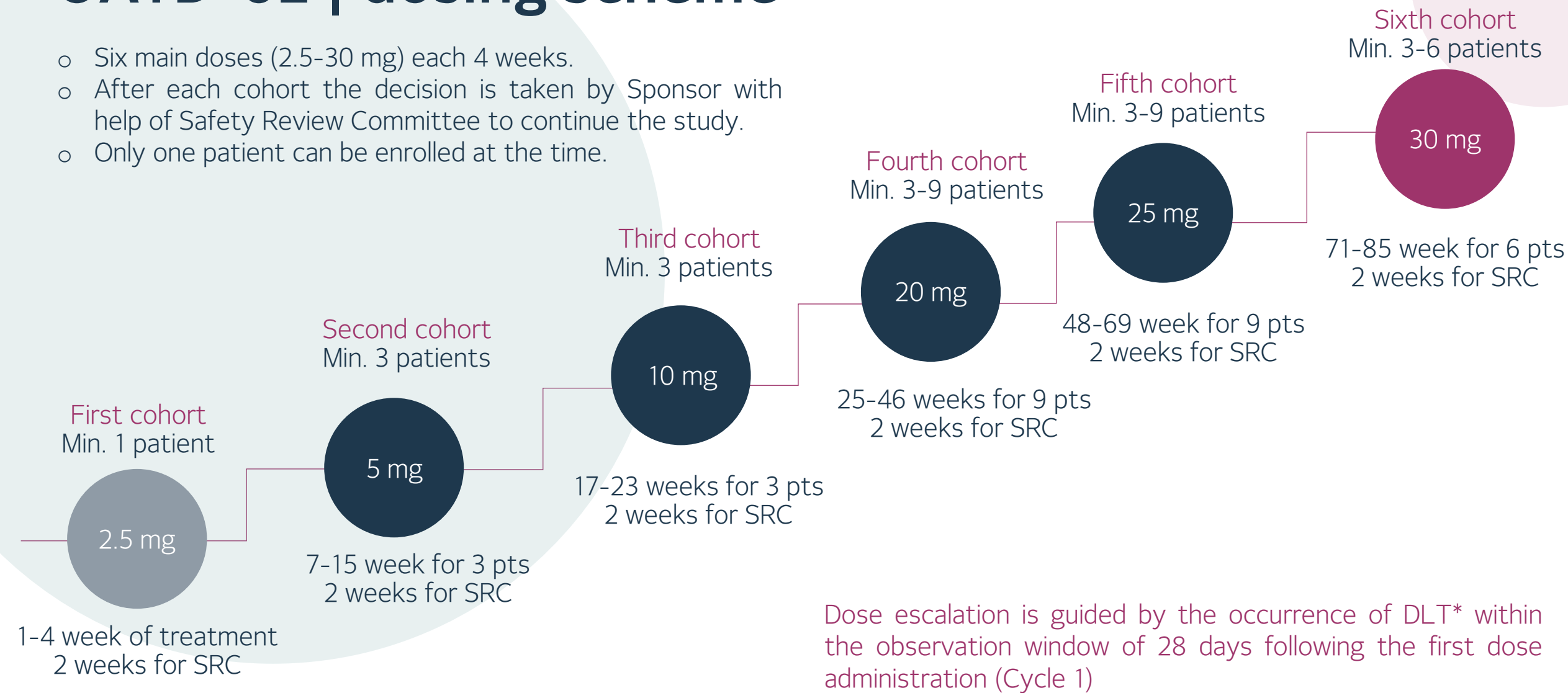


Study Duration: Approx. 1,5 years (Q1 2023 - H2 2024)

Initial clinical data expected at the end of 2023

OATD-02 | dosing scheme

- Six main doses (2.5-30 mg) each 4 weeks.
- After each cohort the decision is taken by Sponsor with help of Safety Review Committee to continue the study.
- Only one patient can be enrolled at the time.



A background image of a scientist in a white lab coat and safety goggles, using a pipette to transfer liquid into a test tube. The image is overlaid with a semi-transparent blue circle. Various laboratory glassware, including Erlenmeyer flasks and test tubes, are visible in the background.

DUBs platform

USP7 inhibitor - a T cell modifier with proven anti-cancer potential

Validated translational potential

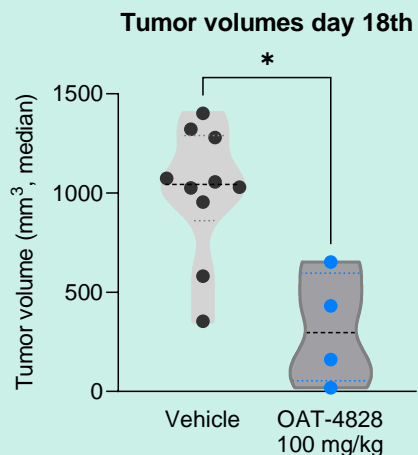
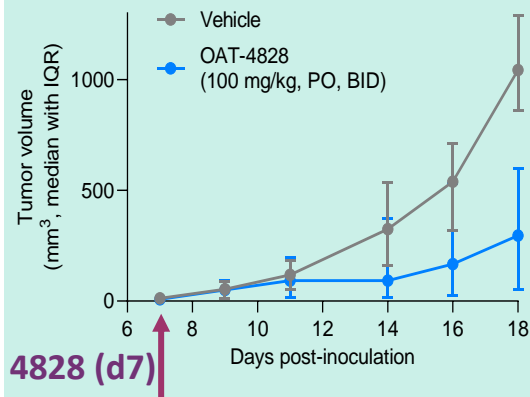
Significant anticancer efficacy as monotherapy and in combinations

Mechanism of action involves activation of T cells

Current focus in the program: optimization of drug-like properties of a lead compound

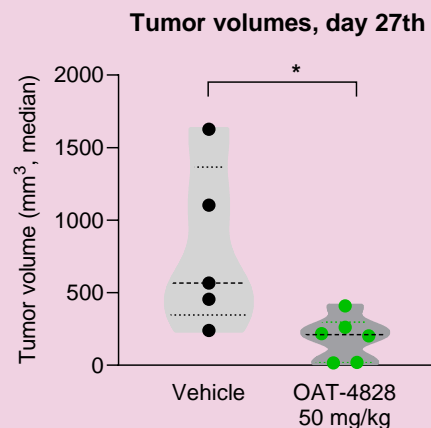
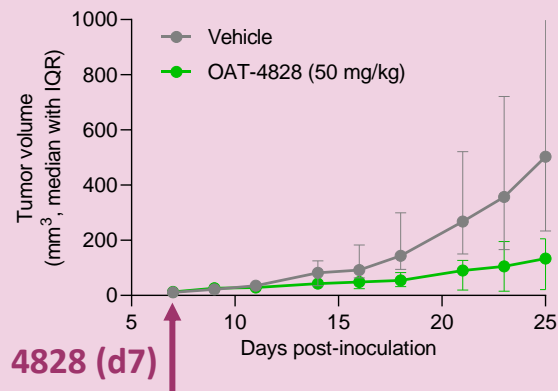
USP7i developed as a tumor-agnostic therapy

B16F10 melanoma



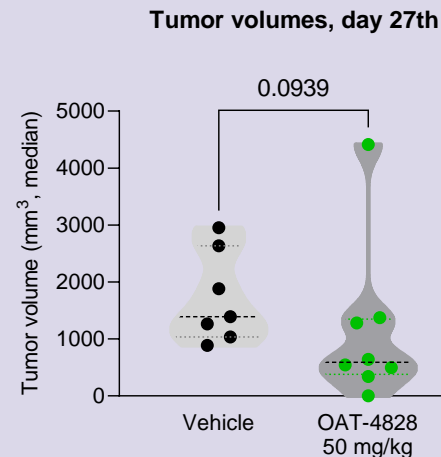
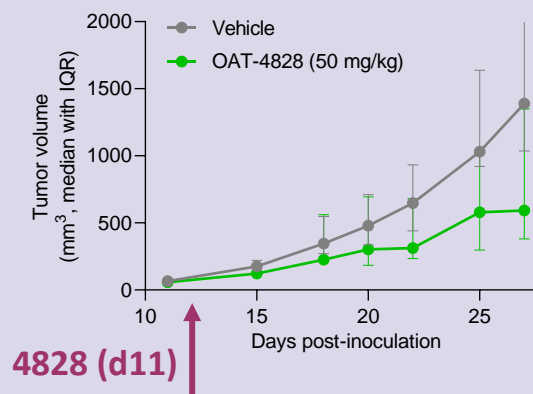
TGI* 72% (100 mg/kg)

E0771 breast



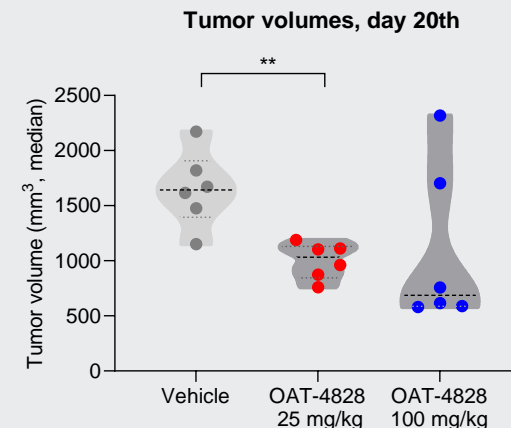
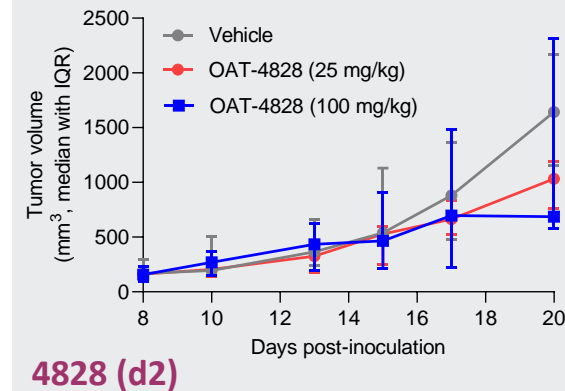
TGI* 62% (50 mg/kg)

A20 lymphoma



TGI* 57% (50 mg/kg)

CT26 colon



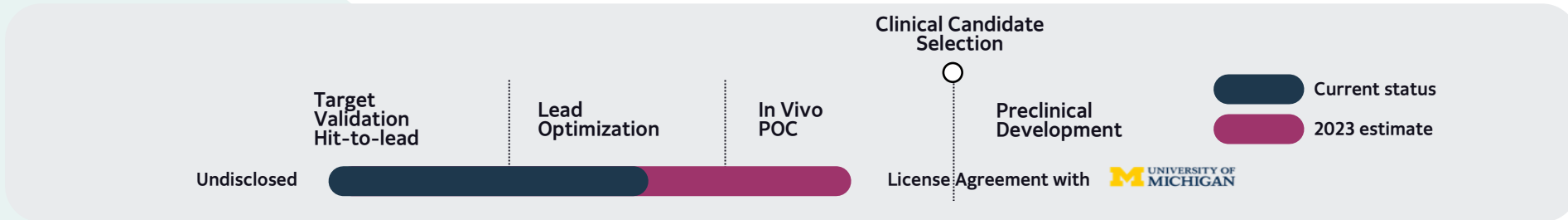
TGI* 37% (25 mg/kg)
TGI* 58% (100 mg/kg)

USP21 inhibitors developed as inhibitors of tumor metabolism



- Launched in August 2021
- Hit molecule found as a starting point for the development of a first-in-class inhibitor of USP21
- Extensive biological studies **validated role of USP21 in cancer metabolism**: USP21 enhances proliferation and migration of cancer cells - we have identified molecular mechanisms involved in this process **demonstrating regulation of known undruggable proteins by USP21** (to be published)
- Screening cascade is developed to study newly synthesized inhibitors

Undisclosed signaling pathway crucial to fibrosis (UoM license)

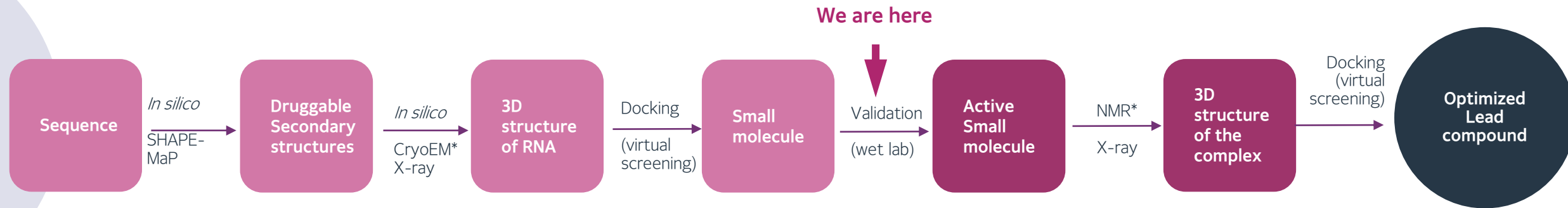


- Launched in March 2022
- The medicinal chemistry team focused on the synthesis of new compounds to create our own IP space
- In February 2023, the Company signed a non-exclusive license agreement with Innovation Partnerships of the University of Michigan, covering know-how in the area of discovering new molecules targeting an undisclosed signaling pathway important in the development of pulmonary fibrosis
- In February 2023, Molecure submitted an application to ABM* for funding of this research

A background image of a scientist in a laboratory. The scientist is wearing a white lab coat, safety goggles, and a face mask. They are holding a pipette and dispensing liquid into a small container. In the foreground, there are several pieces of laboratory glassware, including Erlenmeyer flasks and test tubes, some containing liquids. The image has a blue and purple color overlay.

mRNA platform

mRNA platform | discovering medicines of the future



- Continued development of this novel platform, with a range of analytical techniques being applied to assist structural biology studies of RNA
- In-house investment in both cellular and molecular screening capabilities
- Ongoing collaborations with global leading RNA centers to further leverage the company's expertise and alternative approach to identify compounds interacting with selected mRNA regions
- Service offering developed that will provide commercial partners, involved in the discovery and development of small molecules, with a profit-sharing option. Under this arrangement potential partners will retain the rights to research results and drug molecules but will share revenue from the commercialization of the final product with Molecure



Financials

Financial Results

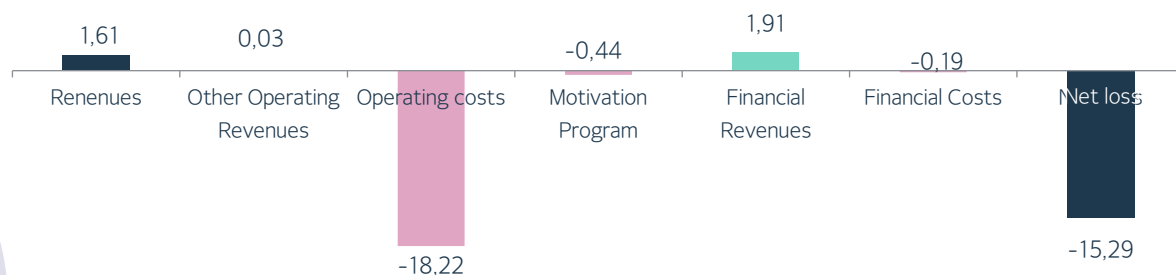
PLN m	2022	2021
Revenue	1.64	1.46
Grants	1.61	1.17
Other	0.03	0.29

Cost incl:	18.22	15.23
General & Adm & Projects*	12.78	9.28
Early stage programs	4.46	0.52
Motivation program	0.44	2.84
Commercialisation costs	0.53	2.59

EBIT	-16.77	-13.76
Net loss/profit	-15.30	-13.64

* incl early discovery programs cost amounted to PLN 4,5 m vs PLN 0,7 m in 2021

2022 Profit & Loss Breakdown (PLN m)



Assets & Liabilities changes (PLN m)



R&D expenses and CAPEX

PLN m	2022	2021
R&D expenses incl.:	25.64	10.10
Arginase program	10.19	2.67
Chitinase program	8.72	4.78
Deubiquitinases & UoM	6.73	2.65
Lab equipment infrastructure**	3.22	1.98
other CAPEX	0.24	0.28
Total	29.09	12.36

*project aiming at an undisclosed signaling pathway crucial for the development of pulmonary fibrosis (licensed from the University of Michigan)

**Incl Lab equipment in Łódź

- Positive decision of the Supreme Administrative Court and corrected ruling by director of KIS indicating expected return of corporate tax of nearly PLN 7m + interest
- Return of PLN 0.4m interest on the received PLN 6m return in August 2022
- Received last payment of PLN of 0.8m from NCBR for the last CHIT1 project

Grant funding

PLN 20m in current grants

Cash position (Mar 2023)

>PLN 53m

Financing secured

Q1 2024



107 employees

50 PhDs

Grants

Submitted to ABM** (Feb 23), NIH** (Mar 20)

ABM
OATD-01
PLN 49/24.5m

ABM
Undisclosed*
PLN 43/29.7m

NIH
OATD-01
USD 2.2m

Planned to PARP** (Apr)

PARP
mRNA platform
PLN 46/29m

PARP
USP21
PLN 57/40m

In total
Submitted 5 applications
PLN 203/133m (over the next 6 years)

*project aiming at an undisclosed signaling pathway crucial for the development of pulmonary fibrosis (licensed from the University of Michigan)

**ABM – Agencja Badań Medycznych, NIH – the National Institutes of Health, PARP – Polska Agencja Rozwoju Przedsiębiorczości

A background image of a scientist in a white lab coat and safety goggles, using a pipette to transfer liquid into a test tube. The image is overlaid with a semi-transparent blue circle. The text '2023-2025 plans' is written in white, sans-serif font across the center of the image.

2023-2025 plans

Potential news flow until Q4 2023

Q2: decisions on one or two ABM grant applications

Q2: FDA approval obtained for OATD-01

Q2-Q3: continued progress in OATD-02 dose escalation

Q3: decisions on the NIH grant for OATD-01 & FENG grants

Q3: First Patient Dosed in OATD-01 clinical trial

Q4: nomination of a clinical candidate in the USP7 program

Q4: PoC confirmed in the mRNA discovery platform

Q4: Maximum Tolerated Dose (MTD) successfully established for OATD-02

Molecule 3-year outlook

Potential milestones targeted by 2025



Protein Targets



RNA Platform



BD/Financial/Operational

2023-2024

- Two clinical assets in PhI/II multi center clinical trials in patients
- Two candidates in formal preclinical development (internal pipeline + in-licensing)
- Three new discovery programs in lead-optimisation

- In vitro PoC reached for 2-3 high value mRNA targets
- Drug-like molecules (leads) in 2 RNA-targeting small molecule programs
- First profit-sharing collaboration on external targets

- At least one clinical stage program partnered in a high-value deal
- High value assigned to the pre-translational & post-translational discovery platform

by 2025

- Final reports from OATD-01/02
- Indication expansion for OATD-01
- One new clinical stage program

- 1-2 additional revenue generating collaborations (revenue+milestones)
- One major partnering transaction

- Significant cumulative revenue from partnering & collaboration agreements
- Dynamic growth: >50% human resources



Thank you

Q&A