



Orphonic Scientific

Turning drug development process into
opportunity **through efficiency**

2021 YEAR-END SUMMARY

FEBRUARY 2022

Agenda

- Introduction – business model and strategy
- Achievements in 2021
- Development plans for 2022 and following years
- Q&A

Orphinic Scientific uses a disruptive business model for R&D sector, recognized by global players



An emerging model for biotech companies could disrupt the R&D landscape and open up new ways to invest, motivate teams, and shape the innovation pipeline.

McKinsey & Company

Disruptive business model

- The Orphinic team makes **strategic decisions** from the Group level, but plays a **key role in managing portfolio companies**
- Portfolio companies focus on developing **single asset**
- We do not invest in facilities or fixed assets – we **fund projects and invest in intellectual property**
- The **diversified projects** in Orphinic Scientific S.A.'s portfolio lower the risk of failure to our investors.

Global comparables

	KEY PEER gossamerbio	elevatebio	ROIVANT SCIENCES	bridgebio	Orphinic Scientific
Discovery	2	0	0	0	0
Preclinical	0	6	3	8	✓ 5*
Phase I	1	1	3	3	✓ 2
Phase II	2	4	10	4	0
Phase III	0	1	1	2	✓ 1**
Value	\$940m	\$845m	\$7.3mld	\$8.0mld	\$121m***

* Includes two medical devices in advanced prototype stage

** Project in pre-commercial stage

*** The \$121m figure is the risk-weighted NPV of the projects in Orphinic Scientific S.A.'s portfolio.

✓ Similarity to key peers

We build our competitive advantage through synergy of competencies of our Scientific Committee members



Adam Kruszewski MD, MBA
CHIEF EXECUTIVE OFFICER

- ✓ Clinical development
- ✓ Exit transactions



Artur Płonowski MD, PhD
CHIEF MEDICAL OFFICER

- ✓ Exit transactions
- ✓ Projects strategy



Jarosław Leszczyszyn MD, PhD, MBA
CHIEF SCIENTIFIC OFFICER

- ✓ Projects strategy
- ✓ Clinical development

The complementary skills of the founders increase confidence in the successful projects execution

Our business model facilitates efficient verification of projects, fundraising for their further development and rapid growth in value

Projects acquisition

Medical DD

Financial and legal DD

Scientific Committee decision

Resource allocation

Project management

Exit transaction

- In 2021 we **analyzed 86 projects** accessed through our global network and use of AI solutions
- **Medical team** verifies project's commercialization potential
- **Legal and financial teams** verify assumptions, valuation and legal risks of the project
- Projects with positive opinions are evaluated by the **Scientific Committee**
- Orphinic holding model allows for flexible financing directly to portfolio companies depending on their capital needs and appropriate allocation of Team Members
- We lead the project to the pre-commercialization stage and then realize profits by selling to large pharmaceutical companies

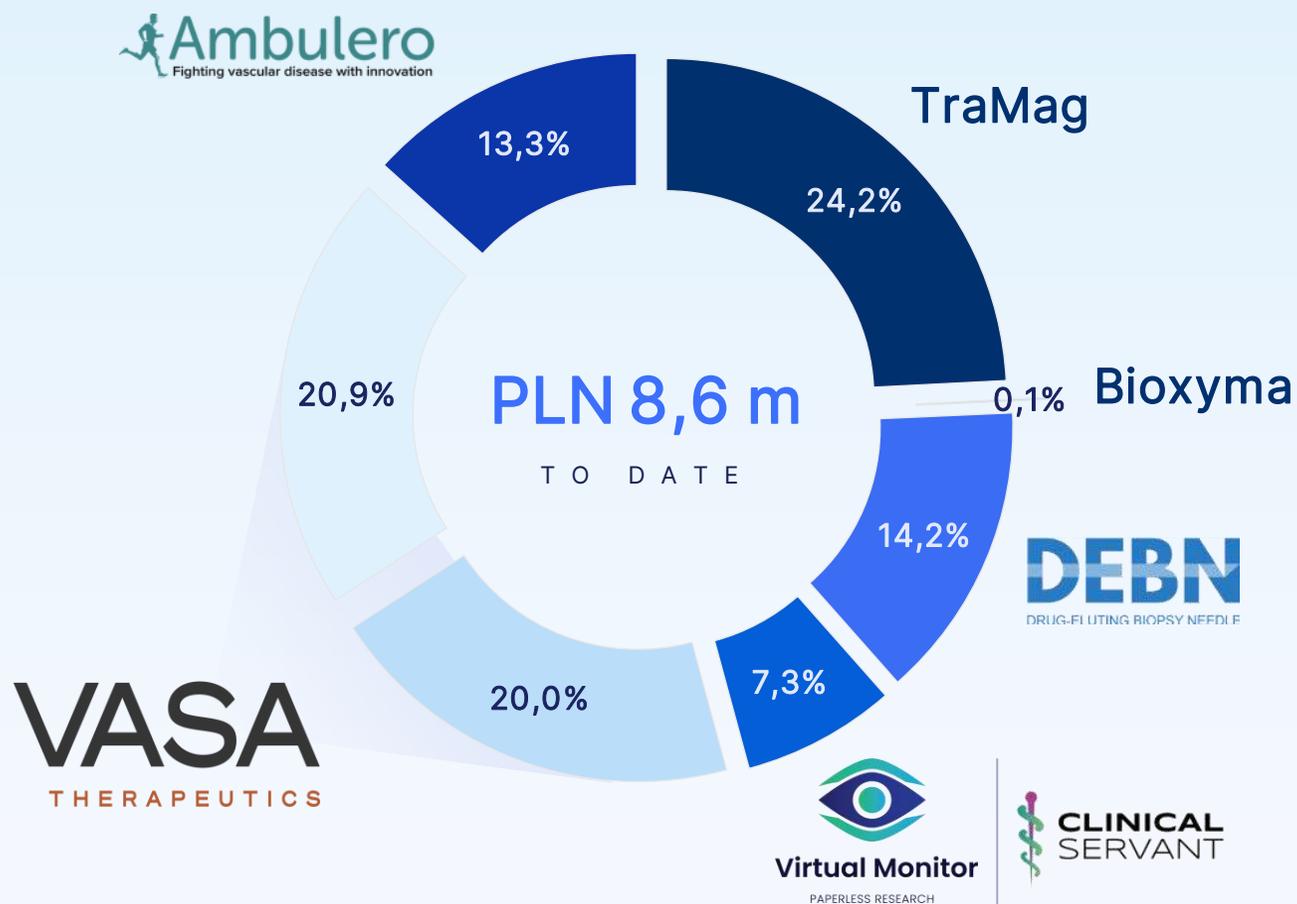
Extended pipeline with first exits planned for Q4 2023 ensures long-term growth of company's value



A world-class team with a successful track record in investment management ensures successful project development...



...with appropriately diversified expenditures on individual projects



1. To date, we have financed the development of portfolio projects for a total of PLN 8,6 m.
2. TraMag and DEBN, projects closest to the exit transaction, required approximately 38% of funds used.
3. Two VASA Programs accounted for approximately 41% of the funds used.
4. The internally developed VMCS project has so far consumed 7.3% of the funds.

Orphinic 2021 in numbers

86

Analyzed projects

3

Newly acquired projects
in the portfolio

7

Projects in the portfolio

12

Target number of projects
in the portfolio business
model

10m+

Capital raised in
subsequent investment
rounds (PLN)

20m+

Public funding¹
in portfolio projects (PLN)

\$121m

Risk-weighted NPV
for the entire portfolio

4

New key operational roles
in the organizational
structure

¹Includes financing from NCBiR, PARP, TISE

We have expanded our portfolio to 7 projects and strengthened our position through collaborations with partners in CEE and in the US

Portfolio expansion to 7 out of 12 targeted projects

- In January 2021, we acquired two strategic projects to our portfolio: **VASA Therapeutics Program 1** and **Ambulero** - a US gene therapy company
- In December 2021, we acquired **VASA Therapeutics Program 2**, a first-in-class drug for peripheral vascular disease.



Cooperation with global partners

- Cooperation with **Google for Startups CEE** provides access to attractive medtech and digital health projects in the region.
- Cooperation with **ArchAngels Fund Chicago** and Washington allows further networking in the US.
- **TriNetX** has become a key technology partner for Orphinic in designing and implementation of clinical trials.



2021 achievements enable the company to rapidly grow with further milestones expected in 2022...

4

New projects in the portfolio – 200 to be analysed



Close cooperation in the development of Virtual Monitor



US market entry and access to capital

Pre-IPO

Raising pre-IPO round in the US



Independent valuation of portfolio projects



Partnering to grow the medtech and digital health ecosystem



Advanced talks with institutional investors from Poland and US



Advancing efforts towards Nasdaq listing

...and two exit transactions planned in 2023

TraMag

Q4 2023
Exit transaction

- Upon PL/EU registration and IND approval in the US, we anticipate an [exit transaction in Q4 2023](#).
- The sales revenue that TraMag will achieve is estimated ~ [\\$186m annually](#), according to independent consultant Bluestar BioAdvisors. |

DEBN

DRUG-ELUTING BIOPSY NEEDLE

Q4 2023
Exit transaction

- Upon completion of Phase II clinical trials in Q2 2023, we anticipate an [exit transaction in Q4 2023](#).
- The sales revenue that DEBN will achieve is estimated ~ [\\$62m annually](#), according to independent consultant Bluestar |

Merger with VASA Therapeutics will transform Orphinic into a global company with a strategic center in the US and an operational center in CEE



1. We have signed a letter of intent with our portfolio company VASA Therapeutics to potentially merge the companies.
2. The transaction will facilitate access to the U.S. capital market and obtaining a higher valuation in the planned pre-IPO financing round.
3. From a strategic point of view, after the merger Orphinic Scientific will become a U.S. company benefiting from a strong network and visibility in Central and Eastern Europe.
4. We have initiated discussions with legal and tax advisors to design optimal transaction structure.

Orphinic Scientific investors participate in the growth of rapidly developing company

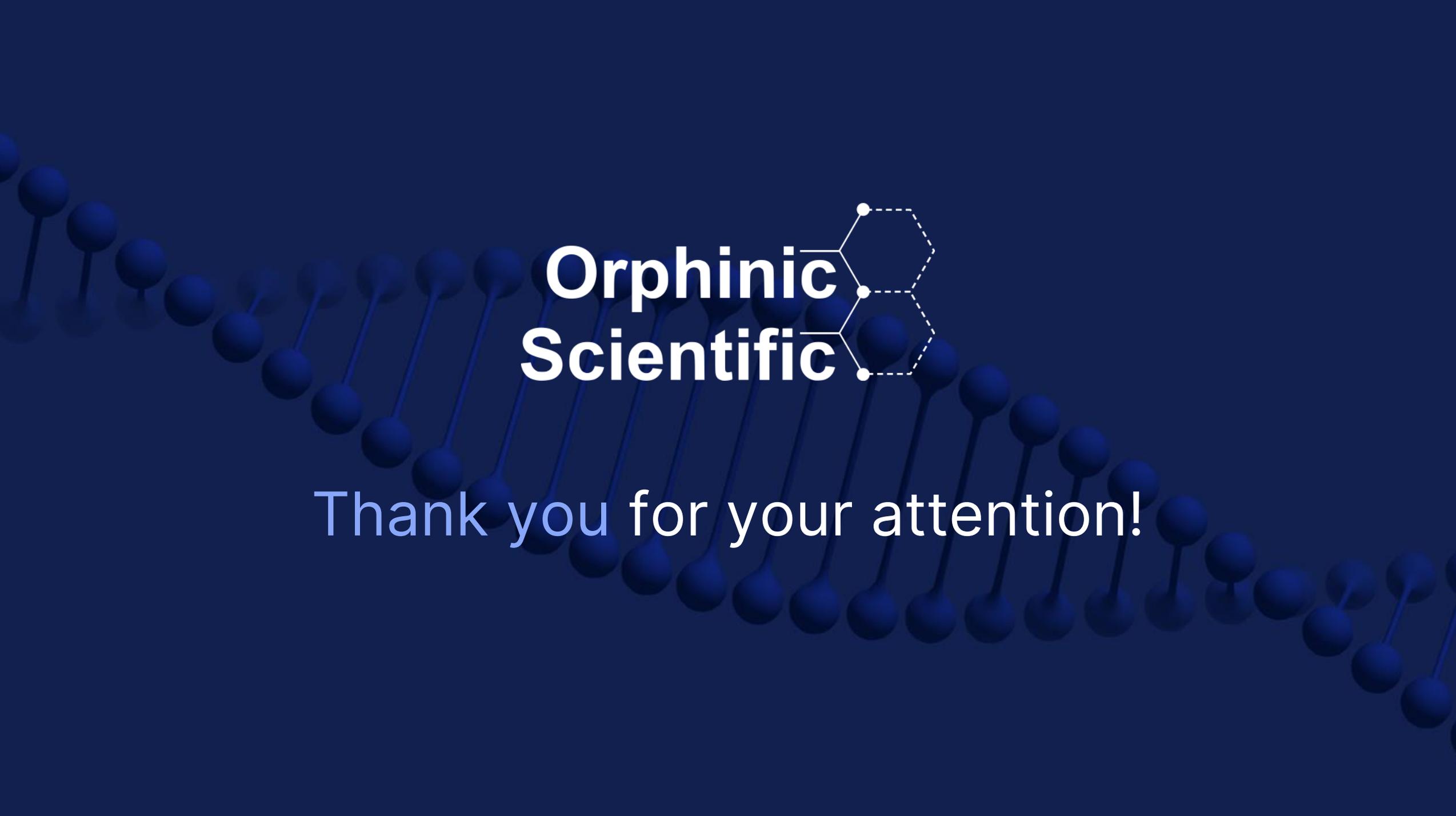
2021 — A breakthrough in building the long-term value of Orphinic Scientific

- Acquisition of a strong project portfolio and creation of a company development strategy
- Creating structure for effective and efficient project management

2022 — Continuation of value growth through:

- Closing of the current PLN40m financing round devoted to the rapid progress of current portfolio projects
- Preparing exit from 2 investments and acquisition of 4 new ones
- Leveraging valuation arbitrage and pre-IPO round in the US market





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Thank you for your attention!



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Appendix

Orphinic Scientific S.A. was established in 2019 as the first Polish biopharmaceutical holding company creating innovative solutions for orphan (rare) diseases and other indications with significant unmet medical needs (Therapeutics, MedTech and eHealth).

Orphinic's core scientific team consists of experts with over 20 years of experience in the clinical trial process and commercialization of medical projects. The Company's clinical development expertise is further strengthened through its partnership with a large CRO operating in Central and Eastern Europe, which facilitates access to diverse patient populations, rapid recruitment for clinical trials supporting our projects and significantly shortens the duration of each phase.

Orphinic currently has a portfolio of 7 projects from preclinical to the market approval stage, in all three healthcare segments: Therapeutics, MedTech and eHealth.

Orphinic is actively seeking new projects through global relationships with universities, think tanks and accelerators, and AI solutions. After acquisition advances them to the commercialization phase





TraMag

- Therapeutic area: **treatment of chronic pain**
- An analgesic drug that allows for a reduced dose of the active ingredient while maintaining efficacy and reducing the risk of side effects

Achievements in 2021

- Advanced registration procedure at URPL and EMA
- Initiation of registration process in the USA
- Development of analytical methods and purchase of active substances for pilot series production

Why did we choose TraMag?

- An innovative product that addresses unmet medical needs in the treatment of chronic pain
- The existing results of clinical trials confirm the therapeutic hypothesis and novel mechanism of action

What will happen in 2022

- Start of production and stability studies (February 2022)
- Registration phase in URPL
- Submission of IND dossier to FDA

Market update

- Estimated US sales according to independent consultants Bluestar at \$186m per year¹

¹Source: Bluestar BioAdvisors report



DEBN

- Therapeutic area: urology and oncology
- Innovative medical device for prostate biopsy with minimal risk of common infectious complications

Achievements in 2021

- Adaptation of the clinical trial protocol to the new recommendations of the European Urological Association
- Product pivot to EMA requirements
- Positive HPLC testing of new formulation and positive testing of packaging substitutions, compilation of technical documentation

Why did we choose DEBN?

- Significantly improve patient outcomes and reduce healthcare costs
- Project advanced at time of purchase on a TRL 5

What will happen in 2022

- Manufacture of coated needles for validation testing
- Manufacture of needles for clinical trial
- Initiation of clinical trials in six centers in Poland and two in other countries (Italy or Hungary)

Market update

- The prostate biopsy market is projected to grow to \$380m by 2020-2024 (CAGR 11%)¹
- More than 4m prostate biopsies are performed each year in the US and EU¹
- Estimated US sales according to independent consultants Bluestar at \$62m per year²

¹Source: American Urological Association market report; ²Source: Bluestar BioAdvisors report



Virtual Monitor

PAPERLESS RESEARCH



Virtual Monitor & Clinical Servant

- Therapeutic area: clinical trial management
- System for automatic monitoring of clinical trials and control of distribution and circulation distribution and circulation of medical substances in clinical trials using a dedicated SCCT (Smart Container for Clinical Trials)

Achievements in 2021

- Filing patent applications and starting international procedure for Virtual Monitor system
- Acquiring funding from NCBiR
- Termsheet with MediVenture
- Establishing close cooperation with CRO Clinmark and partnership with TriNetX
- Developing requirements document, system mock-ups, and assumptions for the tray design and clinical trial process schematics

Why are we developing VM/CS?

- We will reduce CRO costs by c.10% (VM) and revolutionize the way medical substances circulate during clinical trials (CS)

What will happen in 2022

- Development and implementation of MVP VM and TriNetX system
- Construction of technology demonstrators of the SCCT tray for use in a real-world experiment
- Initiation of a clinical trial using the developed systems and device

Market update

- The value of the global CRO market is \$39.6bn¹
- The CEE market makes up about 15% of the global market²

¹Raport rynkowy MENAFN; ²Raport rynkowy PwC



VASA Program 1

- Therapeutic area: cardiovascular system
- First-in-class therapeutic mechanism for the treatment of diastolic heart failure

Achievements in 2021

- Selection of VS-041 compound that meets clinical candidate criteria
- Initiation of large-scale synthesis of VS-041 for therapeutic efficacy and toxicology studies
- Filing of a patent application and work on a second application

Why did we choose VASA 1?

- Unmet need – patients with heart failure with preserved ejection fraction (HFpEF) after first hospitalization for whom there is currently no standard of care

What will happen in 2022

- Evaluation of therapeutic efficacy in animal models
- Toxicological studies
- Preparation of material for Phase 1 clinical trial in 2023

Market update

- The total US market for heart failure therapies is worth > \$40bn¹
- The expenditures of these patients accounts for 2-3% of total health care spending in the United States²

¹Company's own studies; ²Company's own studies



VASA Program 2

- Therapeutic area: **cardiovascular system/muscle mass loss associated with aging**
- A novel therapy for diastolic heart failure. Innovative drug for limb ischemia disease and for diseases that cause loss of muscle mass

Achievements in 2021

- Negotiation and closing of exclusive and global IP agreement
- Acquisition of a portfolio of compounds with high activity and solubility

Why did we choose VASA 2?

- Innovative mechanism of action, attractive pharmaceutical formulation (long-acting drug for once-weekly administration)
- Potentially many additional uses beyond limb ischemia and sarcopenia (e.g., heart failure, kidney failure)
- Significant commercial potential

What will happen in 2022

- Submission of US SBIR grant application (submitted January 2022)
- Advancement of the preclinical studies
- Nomination of candidate for clinical development.

Market update

- Very few drugs in clinical trials, no drug with the same action profile
- Indications have a large market, which is the aging population, with an estimated potential of **\$5bn in global sales annually¹**

¹ Company's own studies

Ambulero



- Therapeutic area: **cardiovascular system**
- First-in-class gene therapy for critical limb ischemia in patients without other conservative treatment options for Buerger's disease or small vessel atherosclerosis

Achievements in 2021

- Advancement of pre-clinical studies
- Obtaining orphan drug designation for Buerger's disease (BD) from the FDA

Why did we choose Ambulero?

- No satisfactory treatment for critical limb ischemia (CLI) that protects against amputation
- The unique AAV construct is patent-protected
- Mechanism of action stimulating formation of collateral circulation confirmed in an animal model and in humans

What will happen in 2022

- Initiation of toxicological studies
- Production of clinical trial material

Market update

- Orphan drug status allows for early commercialization in BD (unplanned phase III)
- Amputation and post amputation care generate the majority of costs in BD treatment (c. \$2bn)¹
- Buerger's disease market is estimated to grow ~3.1% annually (CAGR 2018-2023) and reach \$383m in 2023²

¹Market report Journal of the American Heart Association; ²Market report: Market Research Future



Bioxyma

- Therapeutic area: **pain management**
- Potent analgesic formulation designed to reduce addiction and risk of overdose by chronically ill patients

Achievements in 2021

- Filing an application for funding in the Innoglobo competition
- Conducting analysis of commercial potential in the U.S. market based on feedback from physicians and insurers

Why did we choose Bioxyma?

- Innovative drug for chronic severe pain.
- Bioxyma offers a solution to opioid abuse problem, particularly in the US society.
- Project funded with PLN 10m granny from NCBiR.

What will happen in 2022

- Development of a prototype drug for pre-marketing studies
- Identify active ingredient suppliers and drug manufacturer for clinical trials
- Conduct research for ADF patent and file patent application

Market update

- According to a report published by CDC (2019), 65% of Americans aged >65 suffer from chronic pain¹
- The global cancer pain market generated \$5.3million in 2017. It is projected to reach \$7.5million in 2025, i.e. a CAGR of 4.5% (2018-2025)²

¹ CDC;Report ² BCC Research market report

Portfolio progression in the following years



We plan to maintain a portfolio of 12 projects in the coming years.



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