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An RNA aptamer-based platform technology for the targeted treatment of Cancer

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CEO

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Roadmap

Dec 2020

- Aptadel incorporation
- Patent 1 license
- Interim Management

2021

- Seed investment round closing 1.75M€
- Aptadel Lab set up (PCB)

2022

- Scientific team consolidation

2023

- CEO appointment
- Patent 2 filing

2024

- Patent 1 granted in China
- Lead program: candidate selection
- Bridge round closing 1.5M€

2025

- Regulatory preclinic
- Series A 6M€

2029

- Clinical Trials
- Series B closing 20M€

PROBLEM: Cancer therapeutics are unspecific and highly toxic

- The primary cause of cancer death is metastatic disease
- Metastasis management is performed by chemotherapeutics that kills both cancer cells and healthy cells

TRADITIONAL APPROACH

Chemotherapy

Radiation therapy

- Poor efficacy
- Non-specific
- Serious side-effects
- Low quality of life

FUTURE OF CANCER TREATMENT: TARGETED THERAPIES

- Antitumoral drugs conjugated to delivery agents that takes them specifically to the cancer cells.
- New modalities emerging (e.g. Antibody-drug conjugates, nanoparticles, peptide-based drug delivery)
- Personalized: tailored for specific cancer types and patient subgroups

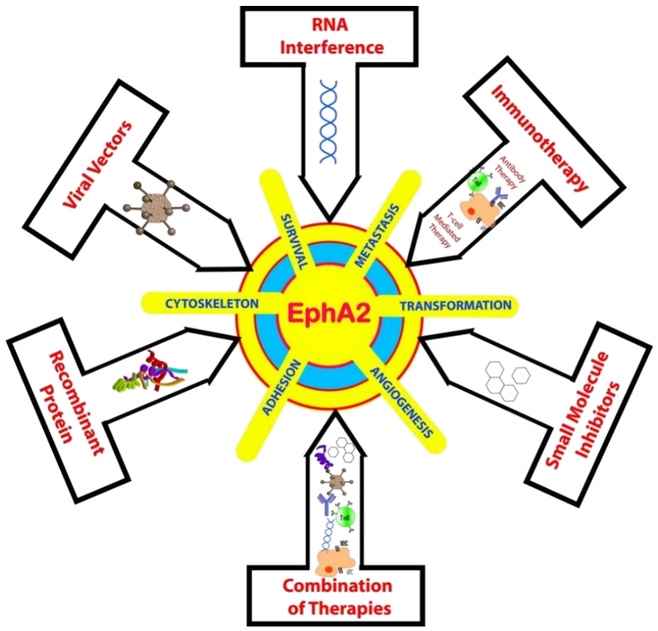
- More efficacious
- Highly specific
- Minimal side-effects
- Minimal effect on quality of life

What if cancer cells can be effectively killed while leaving healthy cells unharmed?

EphA2: a promising target in cancer therapeutics

EphA2

- EphA2 is **overexpressed in a wide variety of solid tumors** and imparts **metastatic characteristics** on previously nonmetastatic cells.
- EphA2 is a cell receptor very attractive as a **druggable target**.



Therapeutic approaches to EphA2

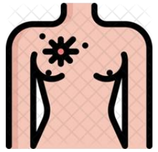
EpHA2 is over-expressed in a wide range of cancer types

Glioblastoma
61% of patients

Ovarian cancer
76% of patients

Melanoma
67% of patients

Colorectal cancer
67% of patients



Breast cancer
92% of patients
0-20% are triple negative

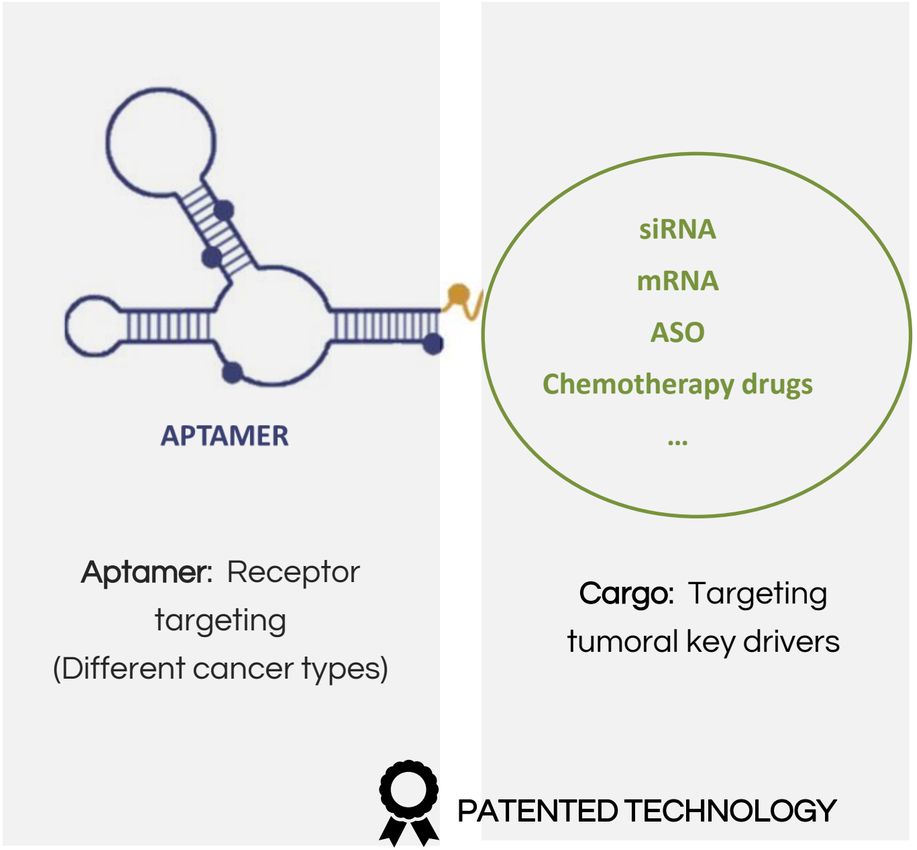
Prostate adenocarcinoma
85% of patients

Sarcoma
90% of patients

Lung cancer
70% of patients

TECHNOLOGY: Aptamer Drug Conjugates

APTADEL has developed an RNA-platform for targeted drug delivery in cancer



ApDCs: Aptamer-drug conjugates
ADCs: Antibody-drug conjugates

	ADCs	ApDCs
HIGHT TARGET AFFINITY / SPECIFICITY	✓✓	✓✓
LACK OF TOXICITY	✓	✓
LACK OF IMMUNOGENICITY	✗	✓
HIGHT TISSUE PENETRATION	✗	✓
COST-EFFECTIVE MANUFACTURING	✗	✓
STABILITY	✗	✓

PLATFORM VALIDATION: Ewing Sarcoma (ES)

WHY

80% mortality in metastasic patients

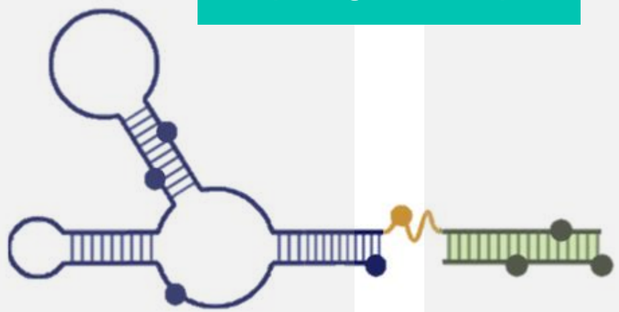
The **available treatments are based on non-specific, low efficacy and toxic** chemotherapy designed for adults.

In the last 30 years, **no new therapies** specifically developed for **Ewing Sarcoma** have been approved by the FDA

Clinical benefits: **Orphan Drug Designation (ODD), Fast Track and Market Exclusivity**

HOW

ADEL-101
(Ewing Sarcoma)



Aptamer: Targeting EphA2
INHIBITS METASTATIC POTENTIAL
(Different cancer types)

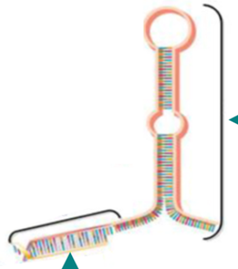
siRNA: Targeting EWS/FLI1*
INHIBITS PROLIFERATION OF ES CELLS

*EWS/FLI1: oncogenic fusion – main driver of ES

PLATFORM VALIDATION: Ewing Sarcoma (ES)

MoA

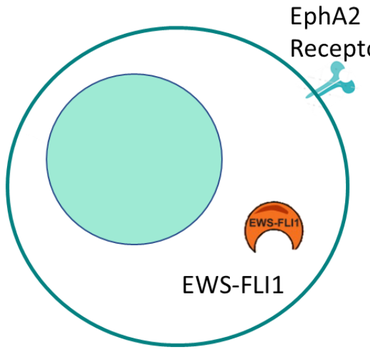
Therapy



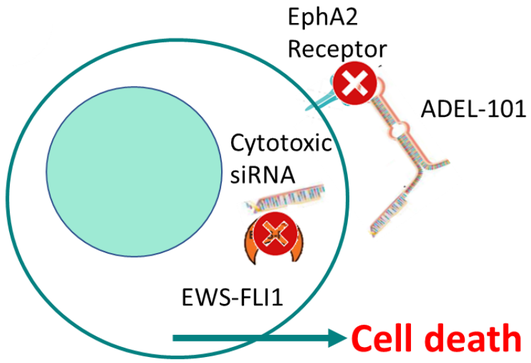
Technology patent protected
(European priority patent)

Aptamer (Cargo Unit)
Selectively targeting cancer cells expressing EphA2 receptor while sparing normal

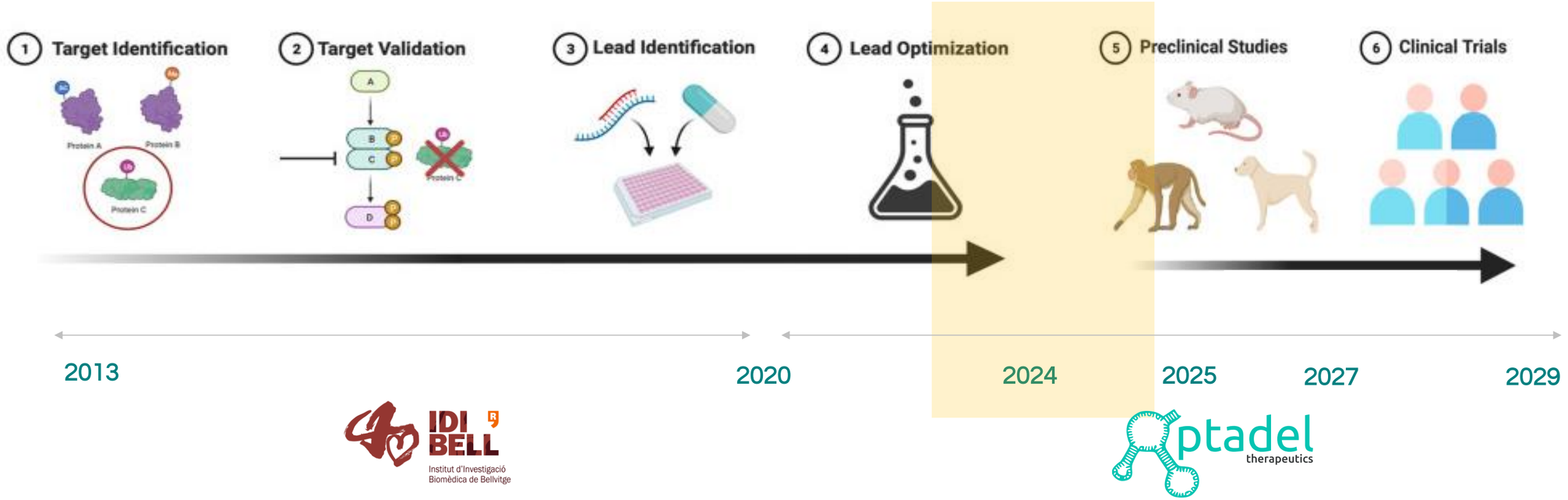
Cytotoxic siRNA
Directed against the Ewing Sarcoma specific transcription factor: EWS/FLI1 fusion protein



ADEL-101 treatment



SCIENTIFIC RESULTS



- Aptamer targets the tumor with specificity
- Aptamer inhibits metastasis in Ewing Sarcoma and Rabdomyosarcoma animal models
- ApDC reduces tumor growth progression and metastasis in ES mouse model
- ApDC has not shown toxic effects in maximum tolerated dose studies in mice
- ApDC shows high stability (in vitro 50% human serum)
- Aptamer antimetastatoc efficacy also demonstrated in vitro in other cancer types (TNBC, pancreatic cancer, metastatic melanoma) – POTENTIAL TO EXPAND TO ADULT INDICATIONS

APTADEL'S PIPELINE STATUS

PROGRAM		DISCOVERY	LEAD OPTIMIZATION	PRECLINICAL	CLINICAL
ADEL-001 (Anti-metastatic drug)	RNA Aptamer				
ADEL-101 (Ewing sarcoma)	Aptamer-conjugate				
ADEL-102 (Rhabdomyosarcoma)	Aptamer-conjugate				
ADEL-103 (Adult indication)	Aptamer-conjugate				
ADEL-201 (Diagnostic kit)	Aptamer-conjugate				

INTELLECTUAL PROPERTY

PATENT 1

Ownership: IDIBELL, F. ALBA and UNIVERSITY OF IOWA

Priority date: June 2019

Status: licensed to APTADEL in exclusivity (2020).

Current stage: granted in China. Under examination in EU, USA, Australia, Canada, Japan.

Covering use in diagnostic and therapeutics of cancer types overexpressing EphA2

PATENT 2

Ownership: APTADEL THERAPEUTICS

Priority date: August 2023

Current stage: PCT filled

Covering use and product (ApDC) in diagnosis, prognosis and treatment of cancer types overexpressing EphA2

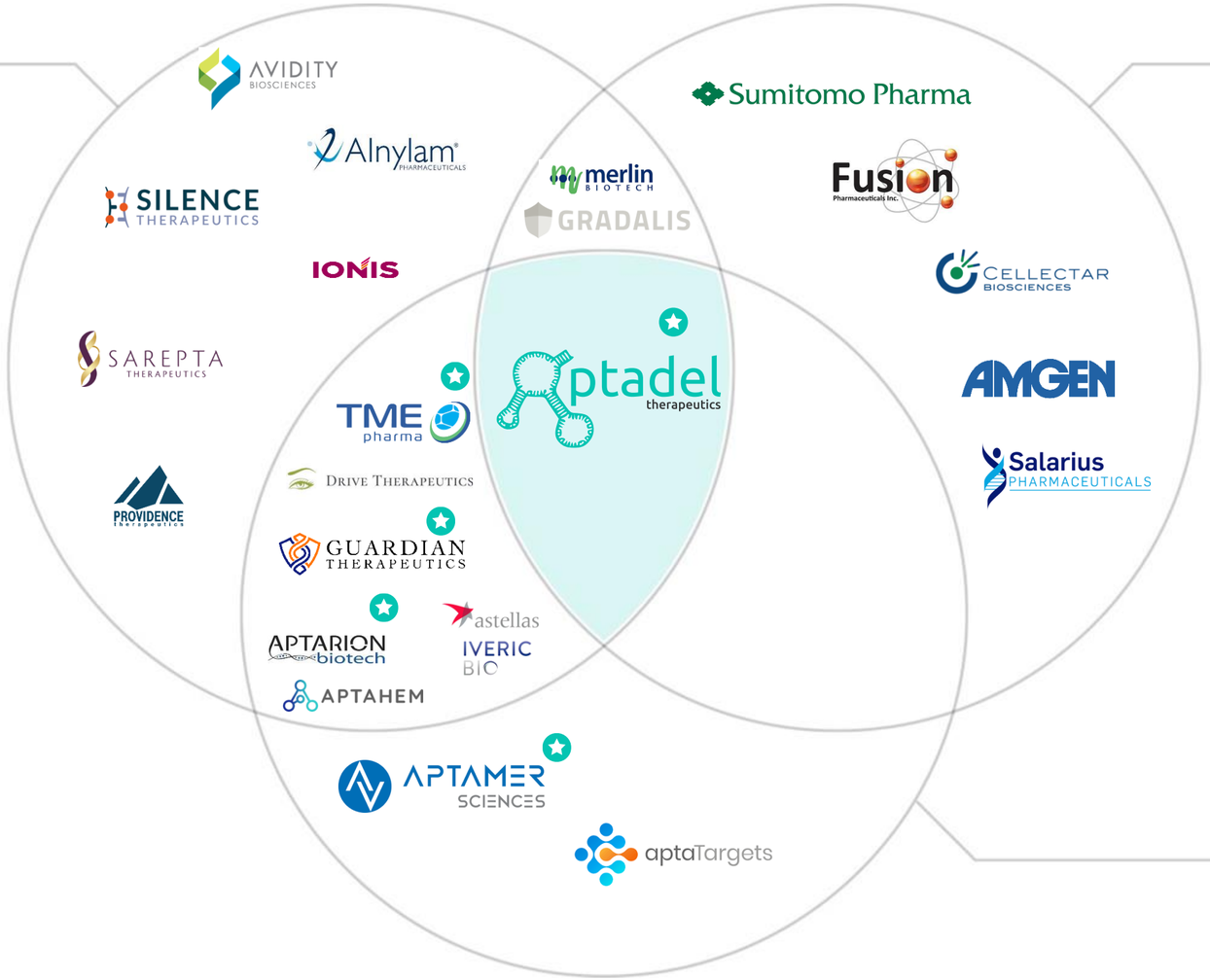
FTO

Positive Freedom to Operate analysis in relation to the commercialization of an RNA-aptamer directed to EphA2 for the treatment of Ewing's sarcoma.

UNIQUE COMPETITIVE POSITION

RNA therapeutics

Ewing sarcoma



EphA2

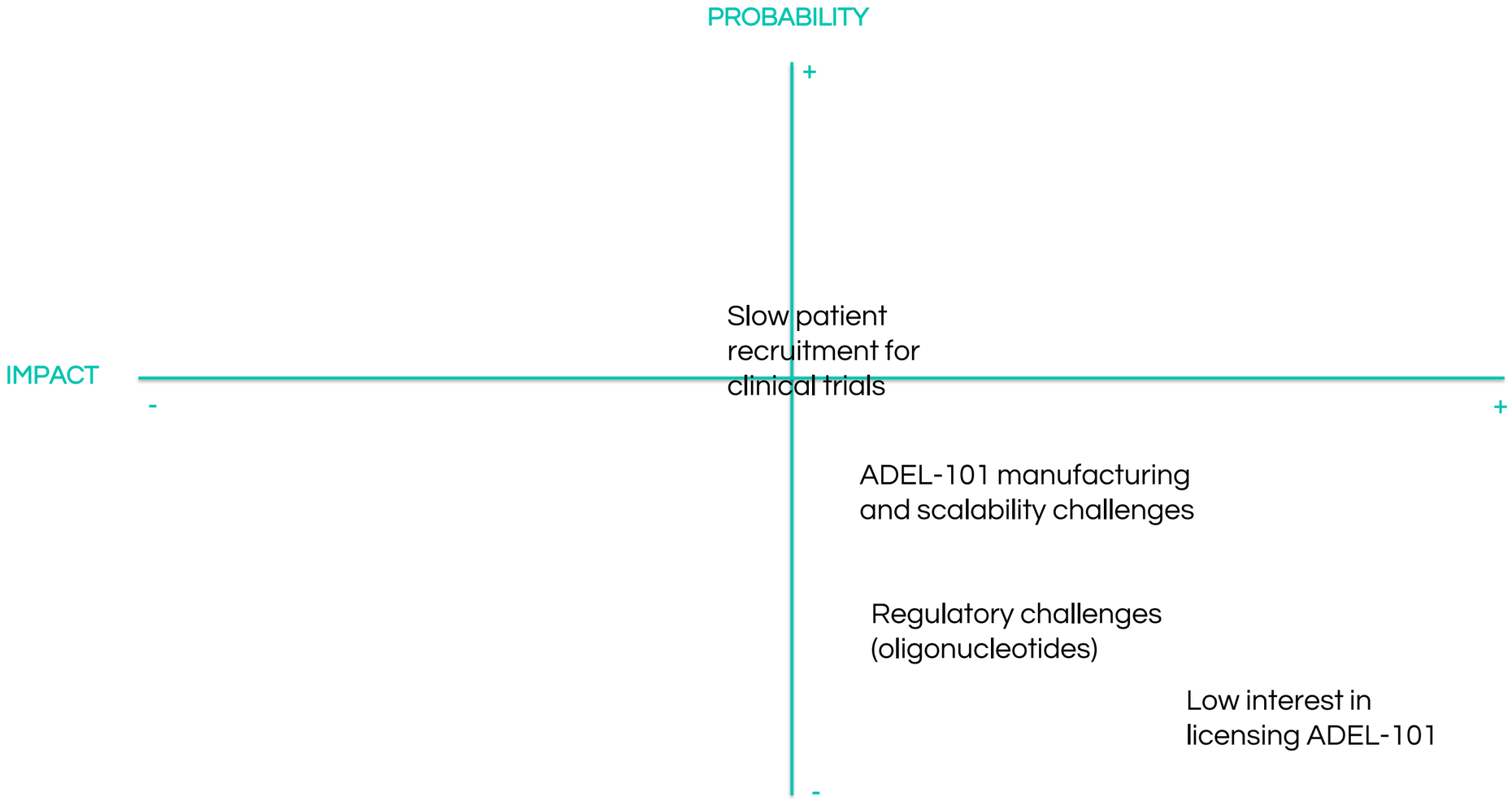
Bicycle®

*Competitor technology targeting EphA2 receptor for a different indication.

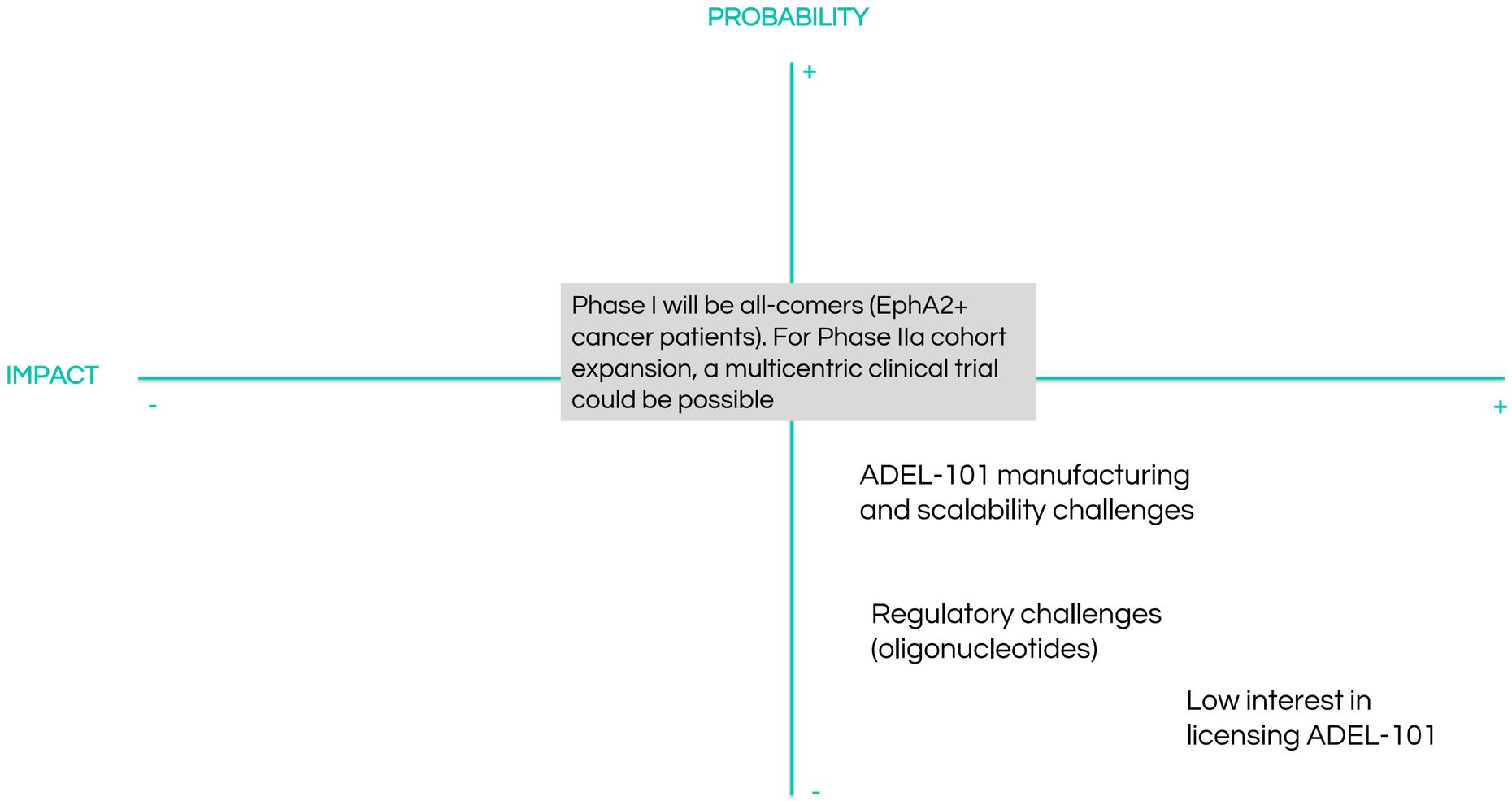
Aptamer technologies

Aptamers targeting oncological indications

PITFALLS AND RISKS



PITFALLS AND RISKS - SOLUTIONS



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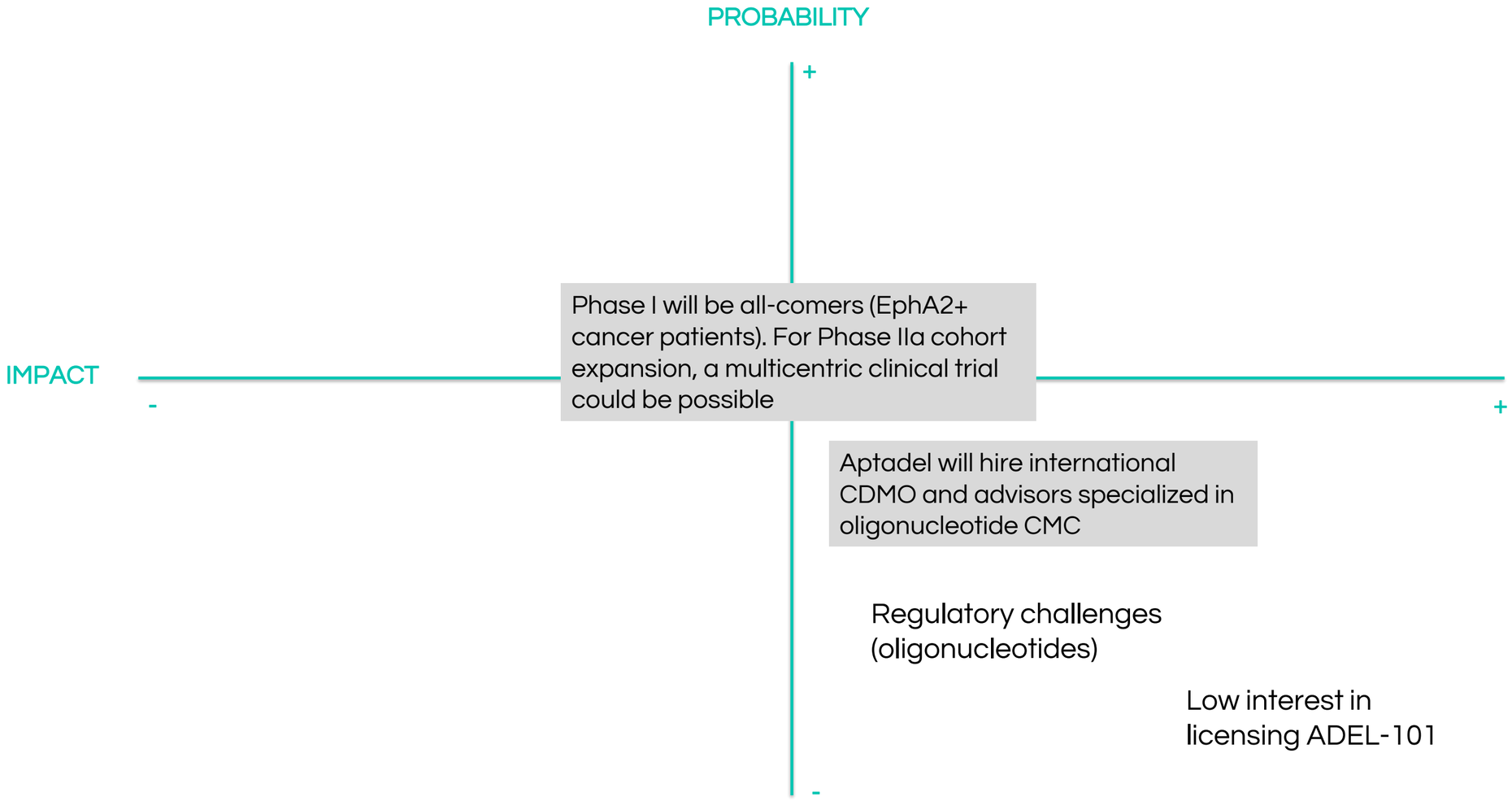

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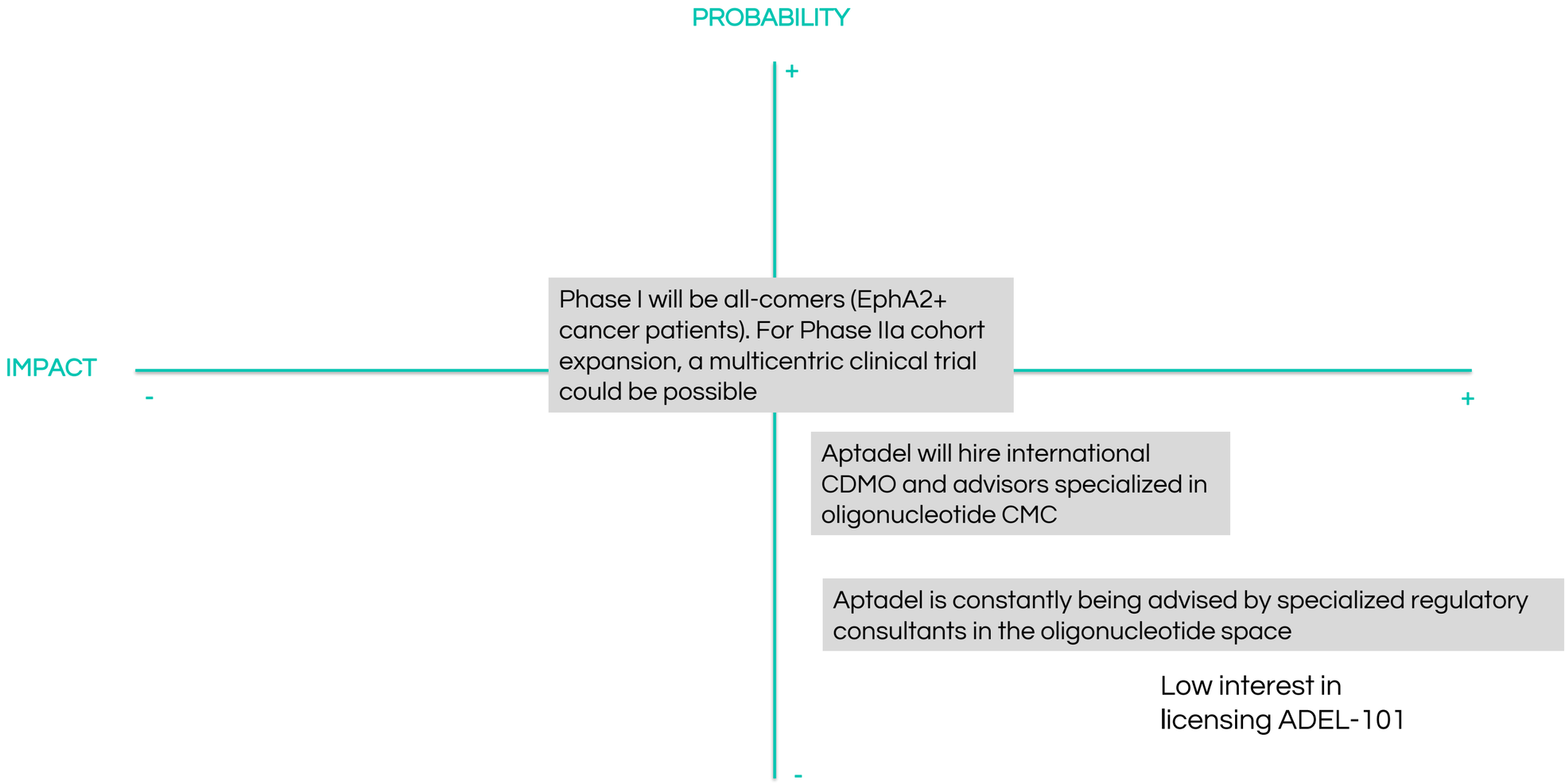

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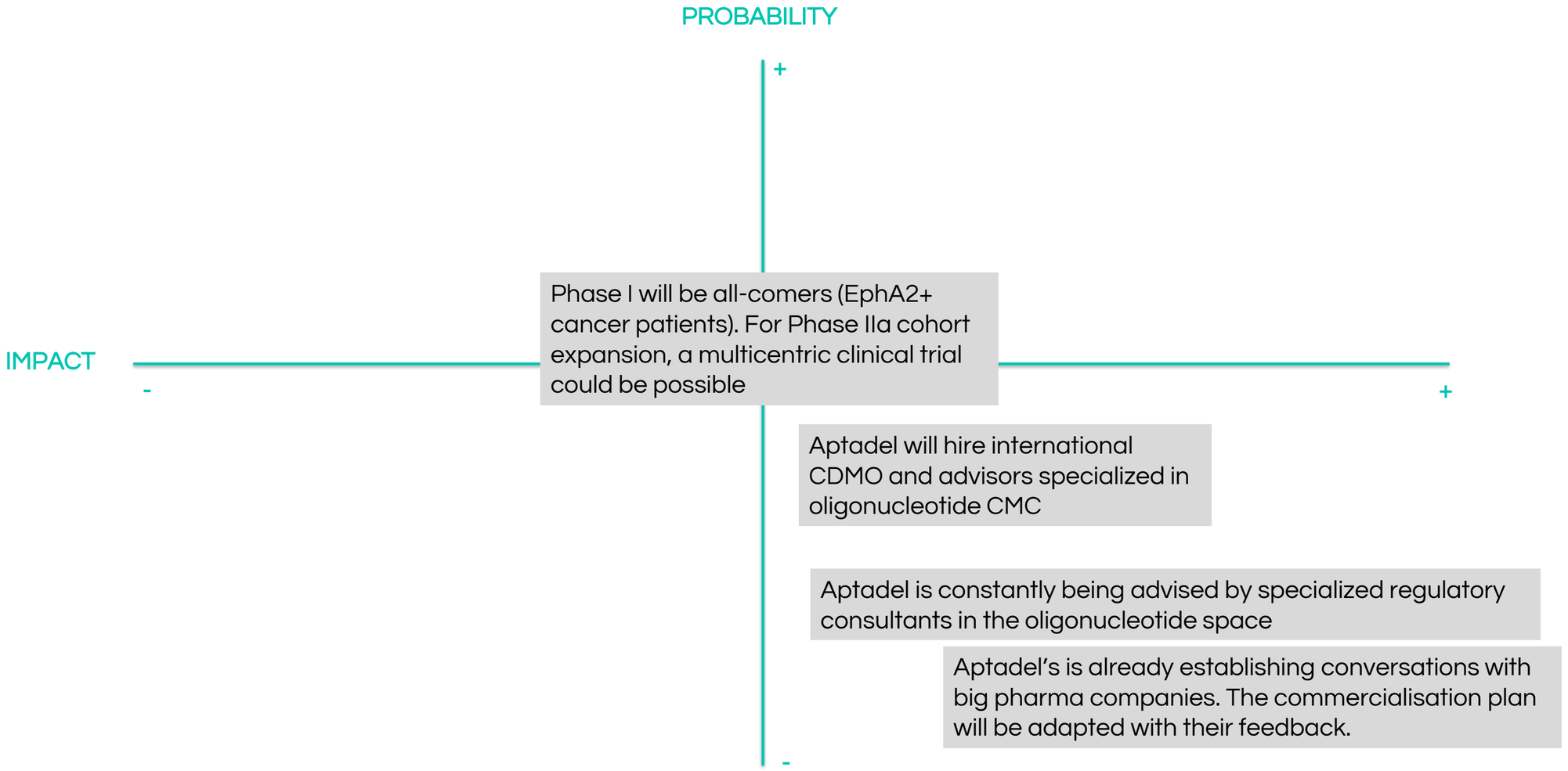
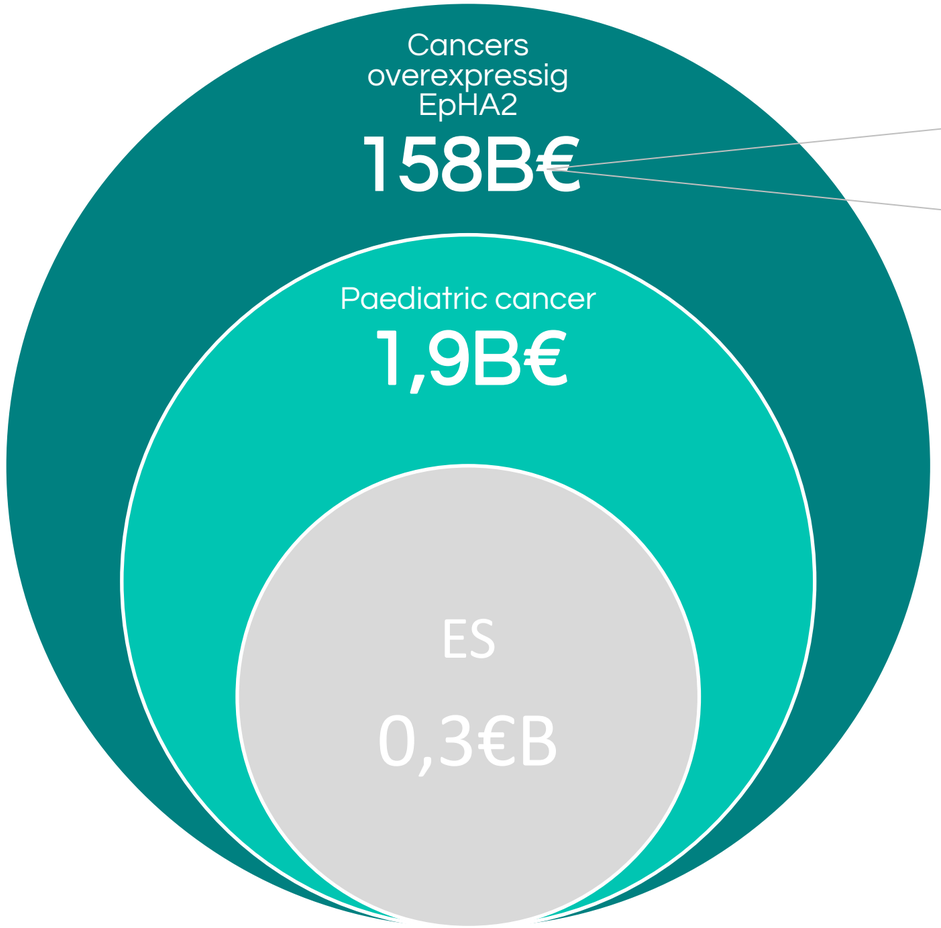


MARKET OPPORTUNITY

The potential of Aptadel's platform for generating **new personalized candidates** for different types of cancer is very high, with a significant impact on the metastatic processes of all solid tumors related to EphA2 and **numerous $B oportunities**



- Prostate
- Breast
- Colorectal
- Cervical
- Melanoma
- Pancreatic
- Bladder
- Bone Sarcoma
- Glioblastoma
- Ovarian
- Esophageal
- Rhabdomyosarcoma
- Ewing Sarcoma

We are looking for a partner that will help us:

Taking ADEL-101 into clinical stage

Expand our product portfolio to adult indications.

APTADEL'S TEAM IS COMMITED TO FIGHT CHILDHOOD CANCER

Gisela Lorente
CEO

Nadia Pons
COO

Oscar Martinez
Academic Founder

Adrian Torres
Head of R+D

Jazmine Arevalo
Senior Scientist & PM

Laura Barbera
Lab Technician

Paloma Giangrande PhD
KOL RNA aptamers drug development

Jaume Reventós PhD, MD
Translational medicine - BOD

Juan Martin Liberal MD
Medical oncology (sarcoma)

Thank you

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aptadel therapeutics