

XXII Encuentro de Cooperación Farma-Biotech

15 de noviembre de 2022

Q2: an aptamer with breast cancer as the lead indication



Miguel Moreno, PhD.
CSO

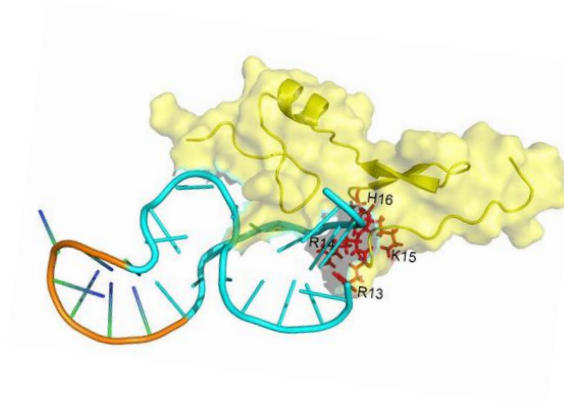
www.aptusbiotech.com

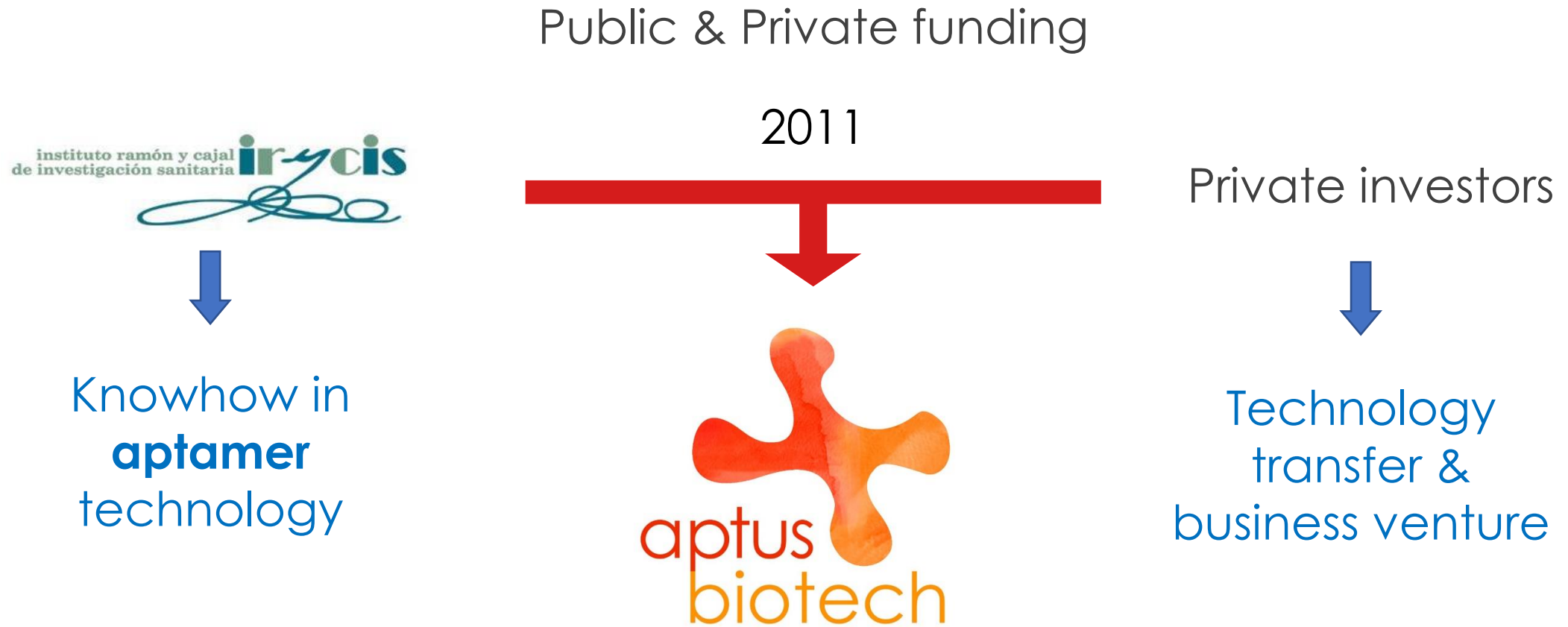
Layout

1. **Aptusbiotech:** Our Mission
2. **Q2: an aptamer targeting MNK1b**
 - a) The problem: **BREAST CANCER**
 - b) Our solution: **Q2**
 - c) Differential features facing the market
 - d) Current status of development
 - e) IPR protection
3. **Partnering Opportunities**

SME devoted to the
Discovery and Development of **aptamers**

Diagnostics and Therapeutics
ensuring the industrial property





Aptamers: a real technology in pharmacology

1. **Nucleic acids** capable of binding to specific target molecules due to the acquisition of a **stable 3D structure**.
2. **Selected *in vitro*** from millions of random sequences to have **high affinity** and **specificity of binding** to its target.
3. Show relevant advantages and are a **clear alternative to antibodies**.

Aptamers' Advantages

Costs

Chemical synthesis

Safety

Non-immunogenic

Long half-life

Reversible denaturation

Range

To a wide number of targets

Other

Smaller size, stability and reproducibility



Specific recognition and stable binding

R&D Services

Selection, optimization and characterization of **aptamers on demand** for our clients

Develop applications based in aptamers

Diagnostics and therapeutics (early stages)

ApTOLL: Aptamer to treat acute ischemic stroke. **Clinical stage phase IIa FINISHED.** Licenced in 2017 to:



Management & Business Development



Ana Seco
CFO



Dr. Víctor M. González
Director & COO



Dr. Miguel Moreno
CSO

Scientific Advisors



Dr. Javier Cortes
Director IBCC



Dr. Elena Martín
Group of Aptamers
(IRYCIS)

Technical Staff



Dr. Gerónimo Fernández
Technical Director & QA



Dra. Ana García-Sacristán
Project Manager



Dr. Celia Pinto
Researcher



Ana Salgado
Researcher



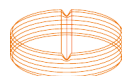
Laura Herraiz
Researcher



Miriam Barragán
Researcher



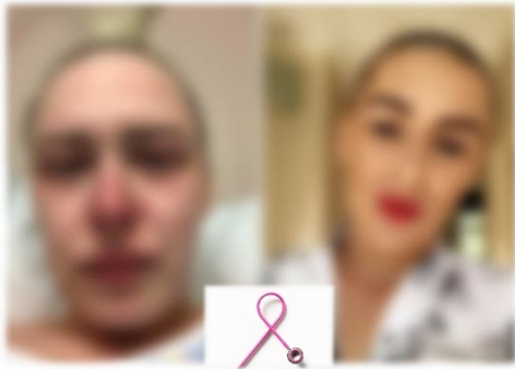
Q2: an aptamer with breast cancer as the lead indication



MEDICAMENTOS INNOVADORES
Plataforma Tecnológica Española

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Breast Cancer: The Problem



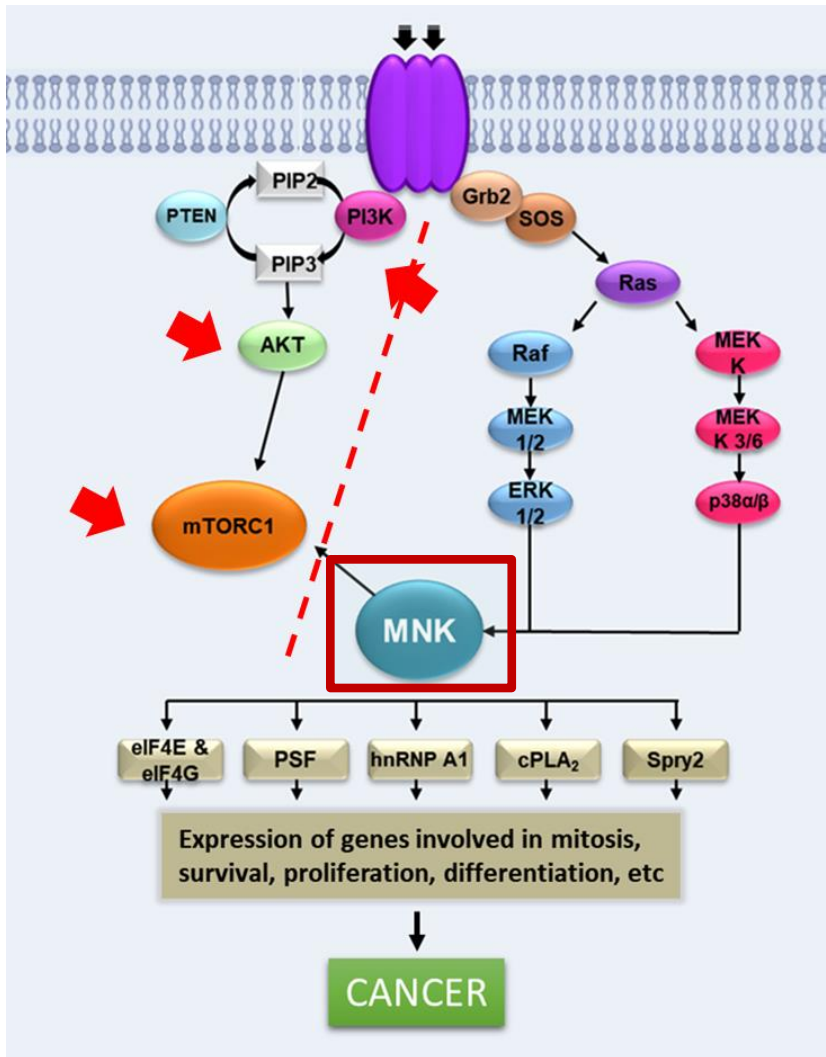
"I lost my mum to breast cancer when I was seven, then I was diagnosed 20 years later" Nicole

<https://breastcancernow.org/about-us/news-personal-stories/i-lost-my-mum-breast-cancer-when-i-was-seven-then-i-was-diagnosed-20-years-later>

- **Highest death-rate by cancer: ~700,000 in 2019**
- **New diagnosed 2.26 million/year.**
- **One of the most commonly diagnosed tumors in women: 24.2% worldwide**

Data: New Global Cancer Data: GLOBOCAN 2020

Breast Cancer: The Problem

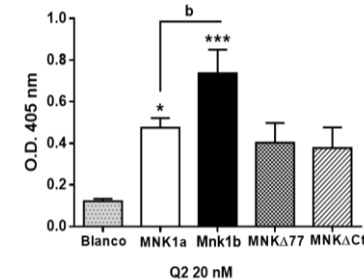


Major clinical problems due to therapeutic resistance

- **PI3K/AKT/mTOR** pathway stand out as **therapeutic target**
- **MNK1b** overexpressed in tumors (not MNK2)
- **MNK1b specific inhibitors** may provide a safe profile and effective anticancer strategy

Q2 is an aptamer targeting MNK1b with breast cancer as the lead indication

- **Q2** is a 29-nucleotide DNA aptamer with **high specificity and affinity to MNK1b**
- **Q2 is protected** by patents filed by the Ramon y Cajal Hospital Foundation (FIBio-HRC) and **exclusively licensed to AptusBiotech**
- **Non-regulatory preclinical**



Kd (MNK1b) = 15,47 ± 2,36 nM
Kd (MNK1a) = 66,13 ± 20,18 nM

Citation: *Molecular Therapy—Nucleic Acids* (2016) 5, e275; doi:10.1038/mtna.2015.50
 Official journal of the American Society of Gene & Cell Therapy All rights reserved 2162-2531/16
www.nature.com/mtna

Characterization of MNK1b DNA Aptamers That Inhibit Proliferation in MDA-MB231 Breast Cancer Cells

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(11) EP 3 663 404 A1

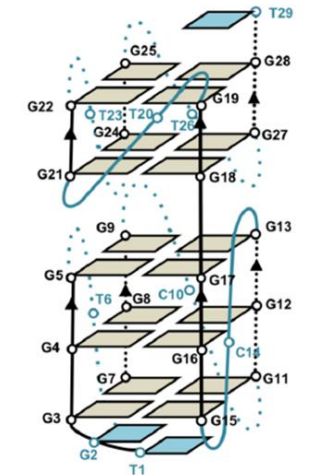
(12) EUROPEAN PATENT APPLICATION

(43) Date of publication:
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C12N 15/115 (2010.01)

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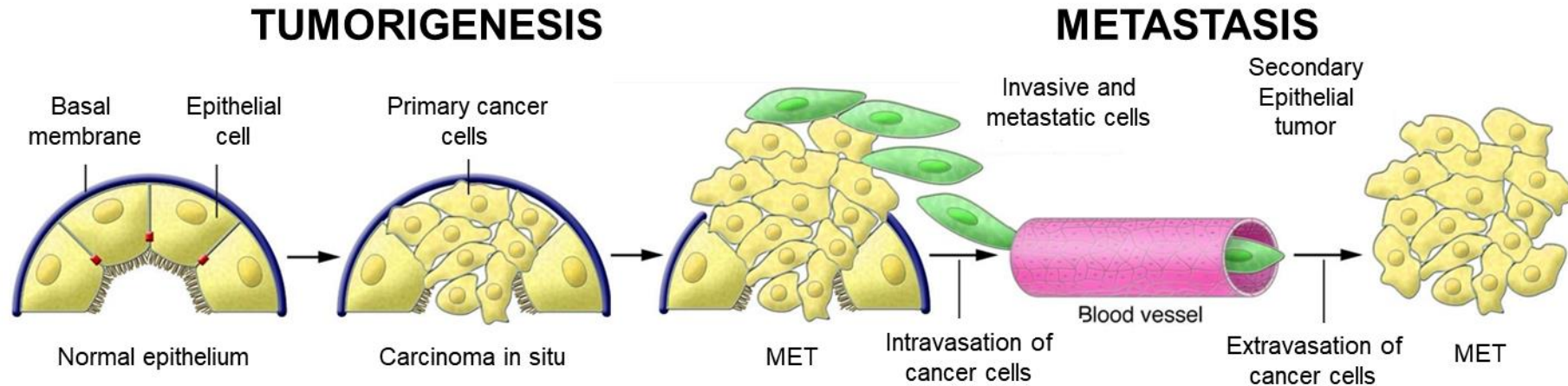
Aptamer Q2



farmaindustria



AptaBreast: *in vitro* results for Q2



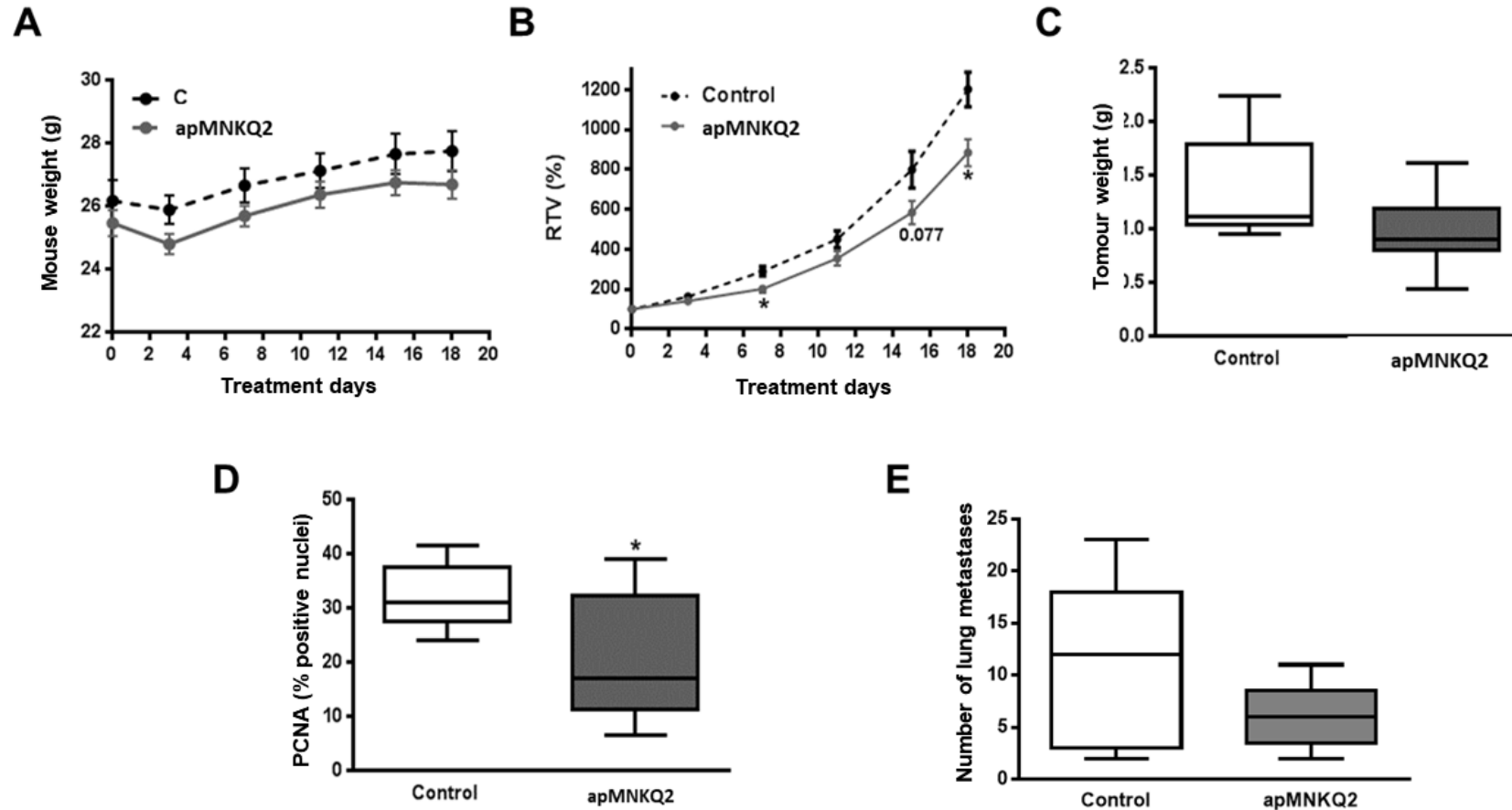
Q2 on tumoral cells

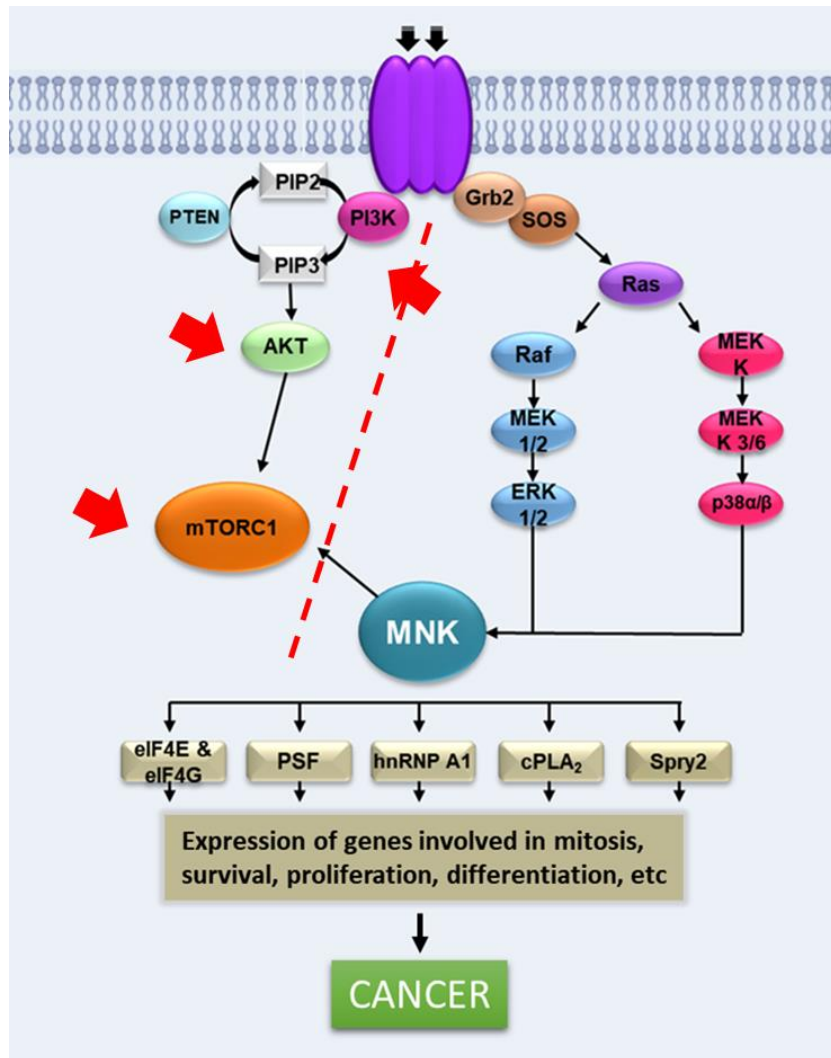
- Inhibits proliferation
- Induces apoptosis
- Inhibits formation of colonies

Q2 on metastatic cells

- Inhibits migration
- Inhibits invasion
- Inhibits epithelial-mesenchymal transition

**Q2 on animal experimental models:
reduces the tumor size, cell proliferation and number of metastasis**

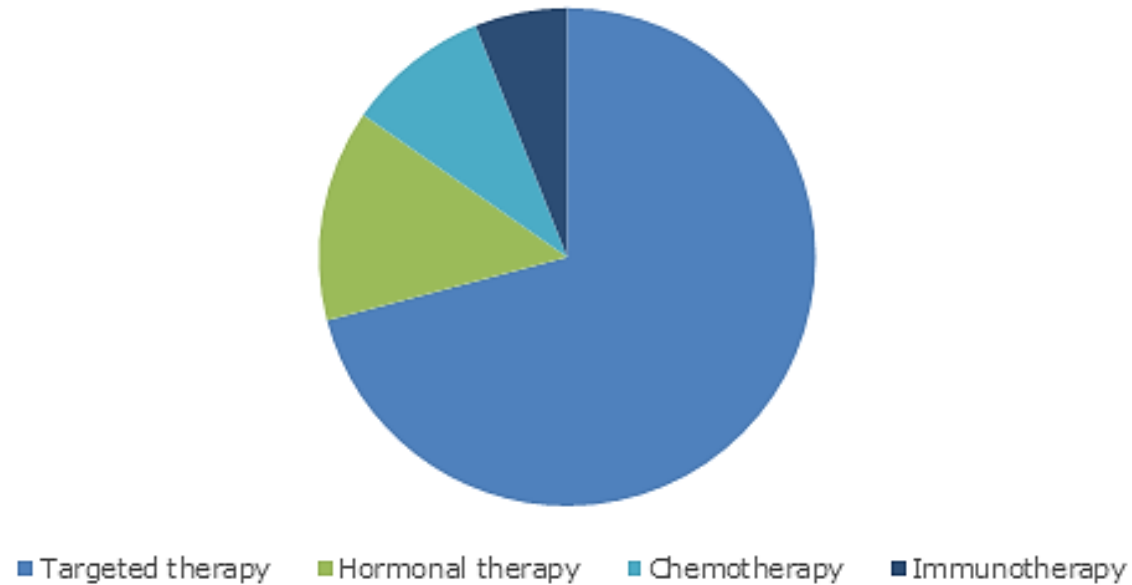




- **No approved drugs** in the same pathway, so far
- **25 related clinical trials** in phases I and II, none of them is with a specific inhibitor of MNK1b.
- Two key processes can be regulated: **tumorigenesis and metastasis**
- Preliminary results show **clean safety profile**

Patent in Europe and USA

Breast Cancer Therapeutics Market Share, By Therapy, 2021



Source: www.gminsights.com

Q2 as a targeted therapy to be used in clinical resistances,
No competes but complete!!!

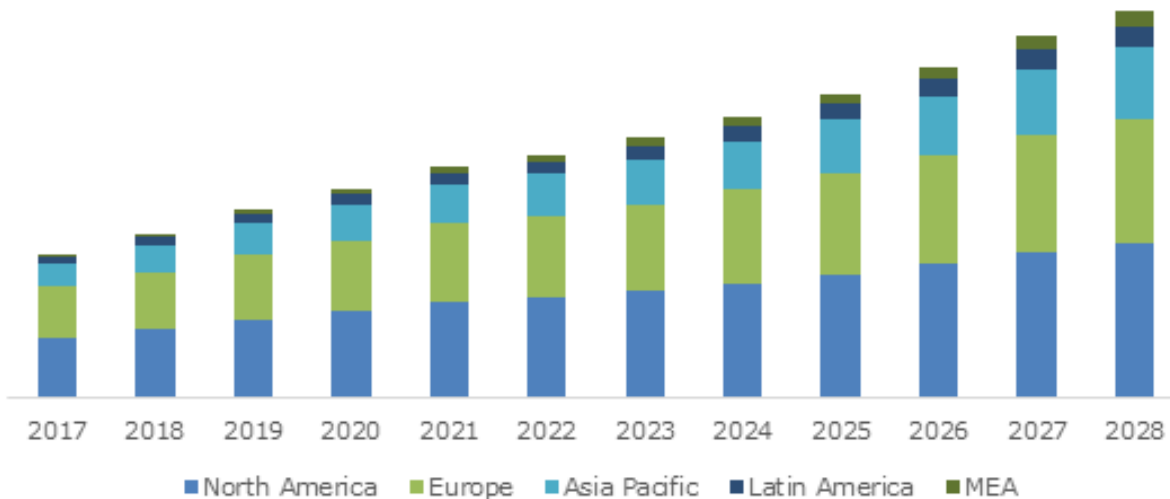
- Patient treatment resistances with targeted therapies :
Q2 as an alternative

- Idea supported by **Dr. Javier Cortes,**

International Breast Cancer Center and involved in pertuzumab, eribuline and **everolimus** development

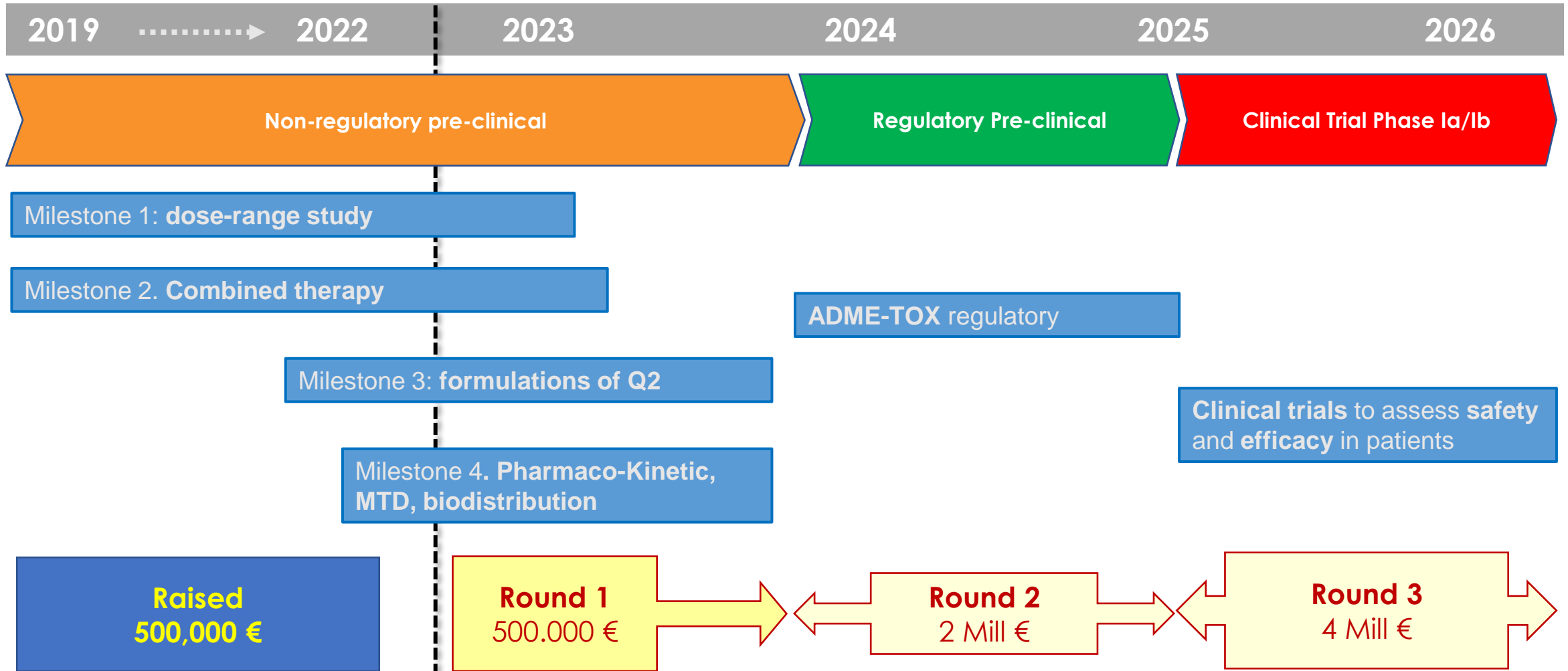


Global Breast Cancer Therapeutics Market, By Region, 2017-2028 (USD Million)



Source: www.gminsights.com

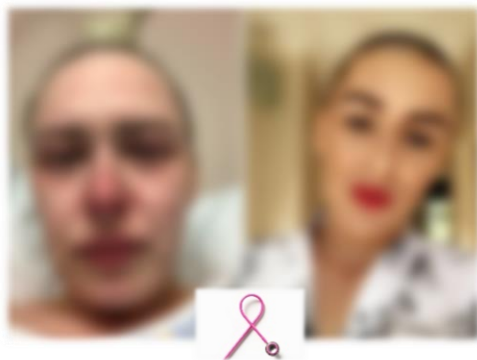
- **CAGR of 8%** per year in the period **2022-2028**.
- Income expected in 2028: USD 42,670.5 million.
- Mammary carcinomas: **€7,800/patient (early stages)** and **€22,000 /patient (advanced stages)**.



License: revenue 70 Mill €



Q2 to deal with **resistances** in **breast cancer therapies**



Let's keep on working!!



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