

# Orphan medicinal products for the treatment of pancreatic cancer

Mulder, J.<sup>1</sup>, Mariz, S.<sup>2</sup>, Van Rossum, T.<sup>3</sup>, De Boer, A.<sup>1,3</sup>, Pasmooij, A.M.G.<sup>1</sup>, Stoyanova-Beninska, V.V.<sup>1</sup>

1. Medicines Evaluation Board, Utrecht, the Netherlands; 2. European Medicines Agency, Amsterdam, the Netherlands; 3. Utrecht University, Utrecht, the Netherlands

## Introduction

- Pancreatic cancer is a **fatal disease** and there exists a high **unmet medicinal need**.
- Pancreatic cancer is a **difficult disease to treat** and many clinical trials fail (Thota R *et al.* Pancreas. 2017).
- Throughout the years, **numerous applications for orphan medicinal product (OMP) designation** were received by the Committee for Orphan Medicinal Products (COMP)

## Research aim

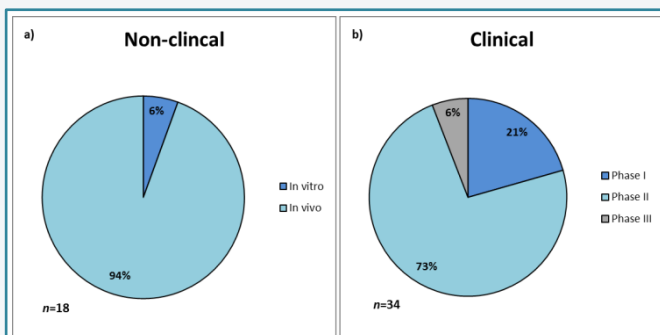
- To **provide more detail on OMP** for pancreatic cancer and **identify if marketing authorisation applications can be expected** in the near future.

## Information sources

- **Internal and publicly available data** from the COMP database were used in this study.
- Information was collected from **summary reports, protocol assistance letters, annual reports** and **public summaries**.

## Results (1)

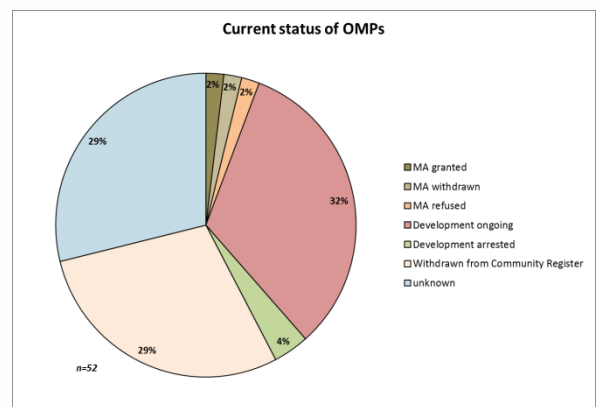
- A total of **80 applications** were submitted to the COMP between 2000 and 2019 and **52 medicinal products** were granted OMP designation.
- At the time of submission, **18 OMPs were in the non-clinical stage** of development and **34 OMPs were in the clinical stage** of development (Figure 1).



**Figure 1.** Stage of development at time of designation. (a) Highest ranked study(ies) conducted in the non-clinical stage of development. (b) Highest ranked study(ies) in the OMPs in the clinical stage of development.

## Results (2)

- MoAs explored included, but were not limited to, **stimulating an immune response** ( $n=12$ ), **blocking signaling pathways** ( $n=8$ ), **inhibiting DNA synthesis** ( $n=5$ ), **infecting tumour cells** and replication therein ( $n=5$ ).
- From the 52 OMPs, **1 OMP obtained a marketing authorisation** and **17 OMPs were still in development** according to the latest annual report.



**Figure 2.** Current status of the 52 OMPs for pancreatic cancer.

## Conclusions

- There is a **high interest from drug developers** as seen from the number of applications.
- At time of application 2/3 of the OMPs were already in the **clinical stage** of development.
- **Various MoAs** are being investigated.
- Drug development is **difficult** with a high failure rate, and to date only 1 OMP received MA.
- For several OMPs development is still ongoing and sponsors still plan to **submit for MA in the near future**.