Experience from sPIP pilot

Industry Stakeholder Platform on RD support 11.07.23

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Launch of sPIP pilot

Initial contact mainly through askEMA

Allocation to PME officer with expertise in the area

Invitation to PDCO member with expertise in the area

Pre-submission meeting arrangements

Requests

12 total

11 through askEMA 1 through paediatric coordinator

First sPIP application received in June 2023 and second one planned for July 2023

Steps

- PDCO decides on D30 if application appropriate for sPIP pilot
- Advice provided during pre-submission meeting on suitability for sPIP pilot

Reasons for potential rejection

- Ongoing PIP
- Previous PIP for the same active
- After the pre-submission meeting it became clear how the development programme could be defined

Therapeutic area

Oncology

Metabolic diseases

Cardiology

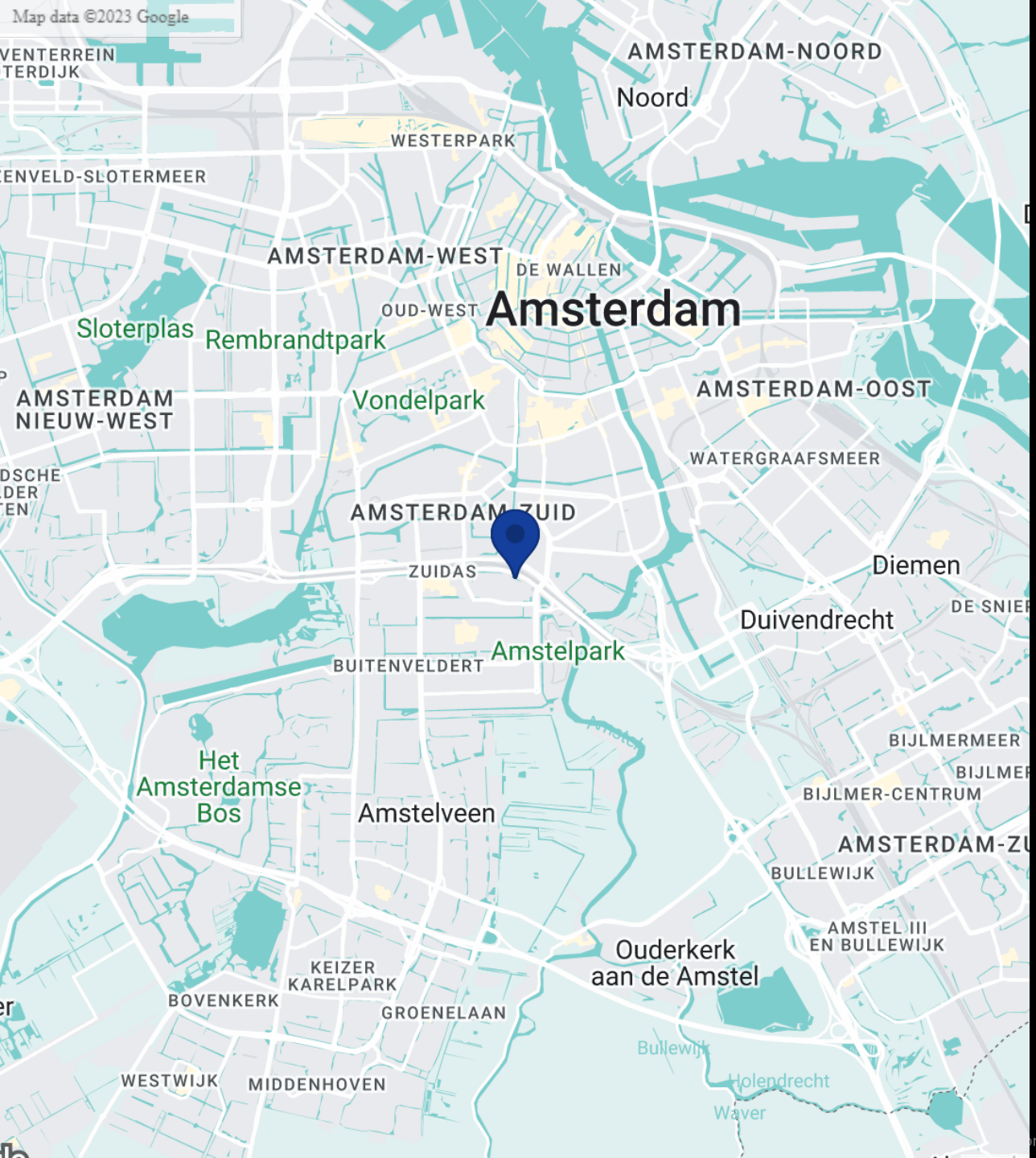
Neurology

Respiratory diseases

Immunology

Reasons for applying

- Data on activity on paediatric cancers need to be generated.
- Uncertainties related to early development (limited toxicology data), and challenges with aspects of clinical development in ultra-rare conditions such as dose finding or endpoints.
- Lack of relevant animal model, no established PD markers, no established endpoints
- Study in infants first in order to define what is required for older children and adolescents.
- Collection of natural history data first before defining the PIP.
- Condition in children different to adults, no proof of concept in humans yet.
- Rare, life-threatening disease, recently identified in children, no approved medicine no regulatory guidance and no validated endpoints.



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