Revision of the key elements and the summary report template

Ongoing revision and proposed implementation PIP Scientific Document B-F – 10th Industry stakeholder platform on research and development support

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An agency of the European Union

Revision of the Key Elements form

It is a **plan**, not intended to be a copy of study protocols

User friendly word format

Resembles **opinion**

Updates to binding elements in studies

Option to use **milestones** in place of M&S analyses if they cannot be defined upfront

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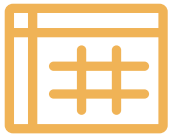
Scientific doc: what's new?

New features

Graphs for development

Leaner structure overall

Focus on NEW Key Elements



New aspects reflected

Embed extrapolation

Updated M&S section

Feed-back from other regulators

Input received from experts

Fully revised formulation and non-clinical sections

Facilitates assessment and input form WPs WGs

More rational Waiver section

Commenting streamlined

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Goals

New agile format

Subparagraphs add flexibility (not applicable option) and guide compilation of the dossier

Cutting down on redundant information

Cut down additional information requests

The new format touches upon key points discussed by PDCO

We expect submissions more fit for purpose

Embed extrapolation concept

Reflection is stimulated on disease similarities adults vs paediatric

New methodologies to have more room

Guidance provided in relevant paragraphs

Clearer info on formulation and non-clinical data

A structured section guides to evidence needed

Focus on what is needed for children

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New Section B core message

Pharmacology and mechanism of action

First in class?

Proof of concept?

Differences and similarity vs adults

Clinical course?

Severity?

Applicability of end-points

Markers?

Unmet need and potential benefit

Formulation

Administration

Safer alternative

Leveraging adult data in the most effective way

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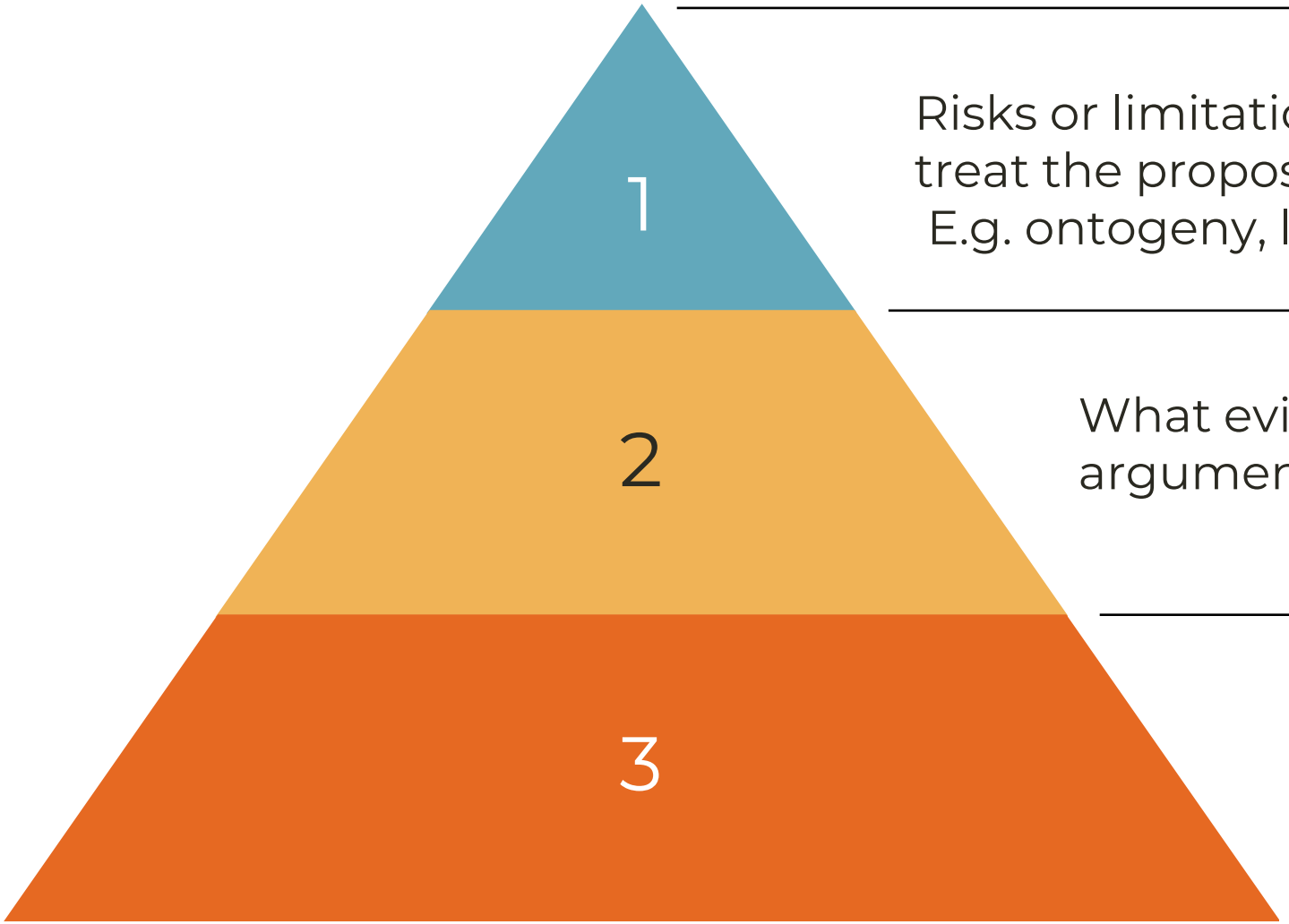
Waivers: expanding on scientific grounds



Risks or limitations of using this medicinal product to treat the proposed target condition in children?
E.g. ontogeny, lack of diagnosis

What evidence is there to support the argument?

Which additional information is needed and limitations to access part of the paediatric population?

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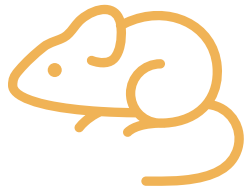
Part D Proposed Paediatric Investigation Plan

D.1. Quality data and proposed development



D.2. Non-clinical data and proposed studies

D.3 & 4 Existing clinical data and proposed paediatric studies

D.4. Other studies proposed & extrapolation plan summary

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What is the formulation proposed?

- Is there a need for a paediatric one?
- Is the adult one adaptable?
- How do we know?

- Components
- Expected volumes
- Expected strenght

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What types of studies have been performed?

Need of additional non-clinical proof of concept data?

Do animal models exist?

Signals from existing data?

Clinical relevance?

Impact on growth and development?

Are studies in juvenile animals needed?

What is the selected species and why?

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Section D.3 structure
Clinical Data

- D.3.1. Existing clinical data

D.3.1.1. Pharmacokinetic properties

D.3.1.2. Pharmacodynamic properties

D.3.1.4. Summary of efficacy data

D.3.1.5. Exposure-response analysis

D.3.1.6. Summary of safety data

- D.3.2. Rationale for paediatric dose to be used in clinical studies

D.3.3.1 Modelling and simulation analyses supporting paediatric development

D.3.3.2. Description of the M&S analysis proposed and role in the development

- POP (PK/PD) Model building methodology and model evaluation.
- PBPK

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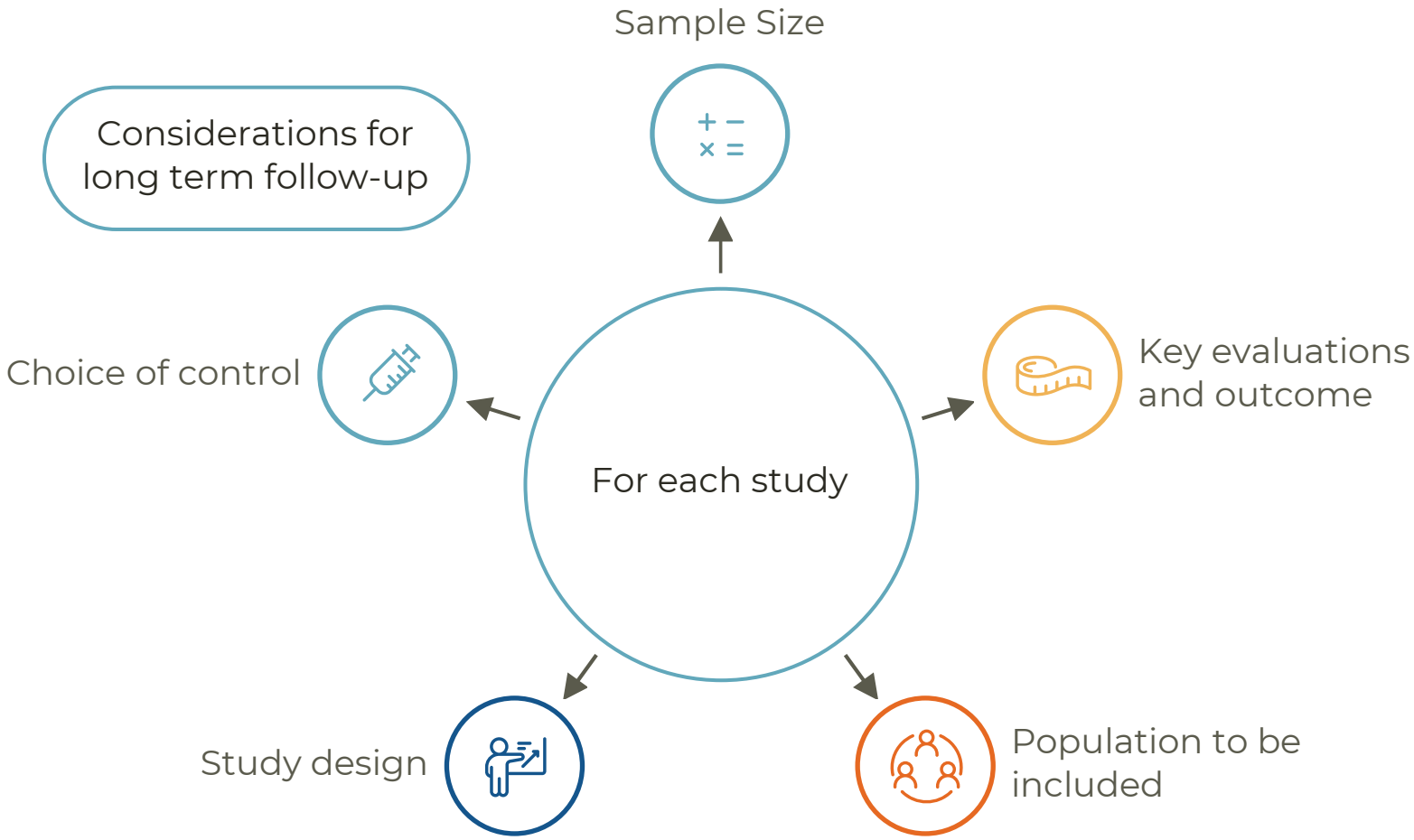
Section D.4 Paediatric Studies



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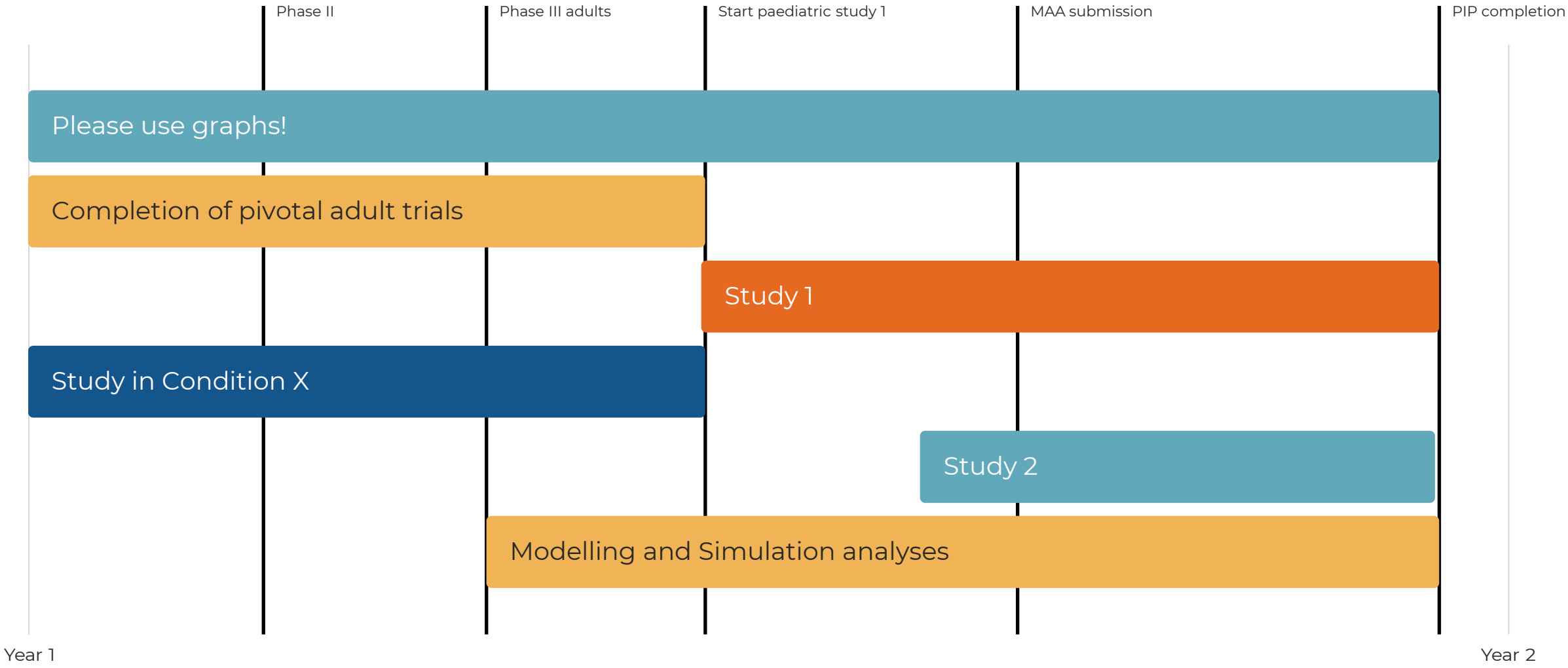
Section E Timelines and deferrals



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Timeline for Scientific Document Revision

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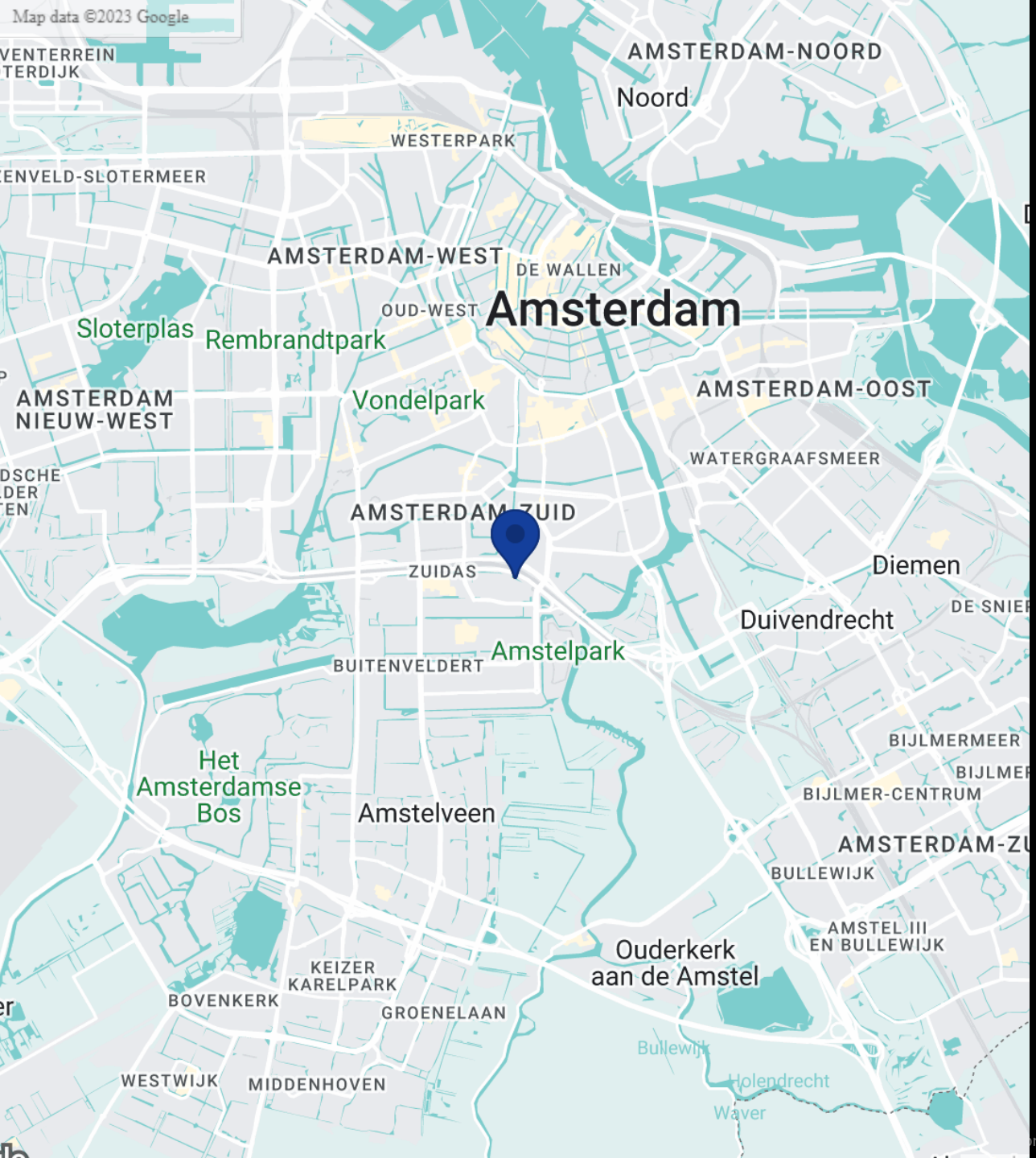

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