Agreeing a Paediatric Investigation Plan with the Paediatric Committee

Paediatric regulatory approval in a nutshell

The paediatric investigation plan (PIP) is a critical component in the development and approval of a new medicine in the EU and UK. It is not possible to file a marketing authorization application (MAA) without one (the agency will reject the submission). This is largely due to a historical lack of paediatric therapeutic options available.

You should plan and submit a PIP early in clinical development (around Phase 1) to reduce the risk of delays to the MAA submission – getting approval for the PIP takes around 1 year.

We have submitted numerous PIPs, modifications and compliance checks across different therapeutic areas and modalities.

Paediatric regulatory requirements to consider

Your PIP should be designed in concert with advice from the Committee for Medicinal Products for Human Use (CHMP). In most cases, the indication for the initial MAA is for use in adults. Therefore, the objective of your PIP is to collect sufficient data to inform on paediatric use (assuming extrapolation of the data is scientifically justified).

In most cases, this involves designing non-clinical, clinical and age-appropriate formulation strategies, which bridge from the adult data for your initial MAA.

It is often desirable to have designed a regulatory pathway to achieve marketing approval in adults, before planning what additional activities might be needed in children. However, if the initial indication for your MAA includes children, then your PIP is similar—to the overall development programme.

The European Medicines Agency's (EMA's) expectation is that your PIP should be submitted following adult pharmacokinetic studies (and prior to starting proof-of-concept or Phase 2 studies), however, later submission is possible, albeit discouraged.

It is usually possible to use deferrals to reduce your PIP deliverables that are due prior to the MAA submission. Sometimes it is also possible to avoid some or all requirements for your PIP with partial, product or class waivers.

If you complete the studies laid out in your PIP, you will be rewarded with a 6-month extension to the EU supplementary protection certificate or a 2-year extension to the orphan exclusivity.

How we can help you get your PIP approved

We have submitted numerous PIPs across different therapeutic areas and modalities.

We can help with the:

- initial strategy and planning,
- drafting the submission,
- addressing the questions from the Paediatric Committee (PDCO),
- making changes through modifications and addressing the pre-MAA compliance check, and
- planning to obtain the PIP reward.

We often help companies develop and align their PIP with the initial paediatric study plan (PSP), which is required by the Food and Drug Administration (FDA).

Your roadmap to working with Somerville Development Partner

Initial conversation

It is always a good idea to start with an open-ended discussion about your needs and challenges, and we can give you some preliminary advice on how to approach your regulatory interactions in Europe and the US.

Project scoping

To provide a specific proposal for how we would approach your project, we require access to some of your project details. Therefore, we always recommend signing a confidentiality agreement before discussing the scope of your project.

Next, we will provide a scope of work for you to review. Once this is agreed, we will start work on the project.

Kick-off

We will assign an experienced consultant to act as your Regulatory Lead from the very beginning—your expert will take overall responsibility and accountability for your project from start to finish.

Your Regulatory Lead will always be someone who has:

- 10–15 years of experience
- tenure in established pharmaceutical and biotechnology companies, and
- been the regulatory lead on a global project team.

Your Regulatory Lead will be your primary point of contact and will also draw on support and expertise from additional consultants, as needed.

We ask that you securely share all product-related documents so that we can conduct a detailed evaluation of the data. This will enable us to provide informed recommendations for how we will deliver the project.

The most common documents we request include:

- study protocols,
- the investigator brochure,
- clinical study reports,
- briefing books from prior regulatory interactions, and
- meeting minutes from prior regulatory interactions.

This also means that we can repurpose as much content as possible to reduce costs.

We will agree the timelines, overall project delivery and desired outcomes with you. We will also identify the relevant stakeholders who will need to review and provide input into the project.

Delivery

During the project, we look out for your best interests and work to help you achieve your corporate objectives. We work as a partner and proactively identify issues, solutions and opportunities, rather than acting as a passive or transactional vendor.

Our consulting team will produce the agreed deliverables to the required timetable.

We will meet with you as often as needed to discuss the progress of the project and request any additional information. We will also meet—as necessary—to discuss review cycles, final signoff and submission.

Every project we work on undergoes peer review, as well as quality control of the final submission. That way we effectively draw on all additional experience from among our team of life sciences experts.

Feedback

Feedback is very important to us, so most of all we want to hear your thoughts on how the project went.

Getting in touch

We always welcome the opportunity to discuss a potential project with you!

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