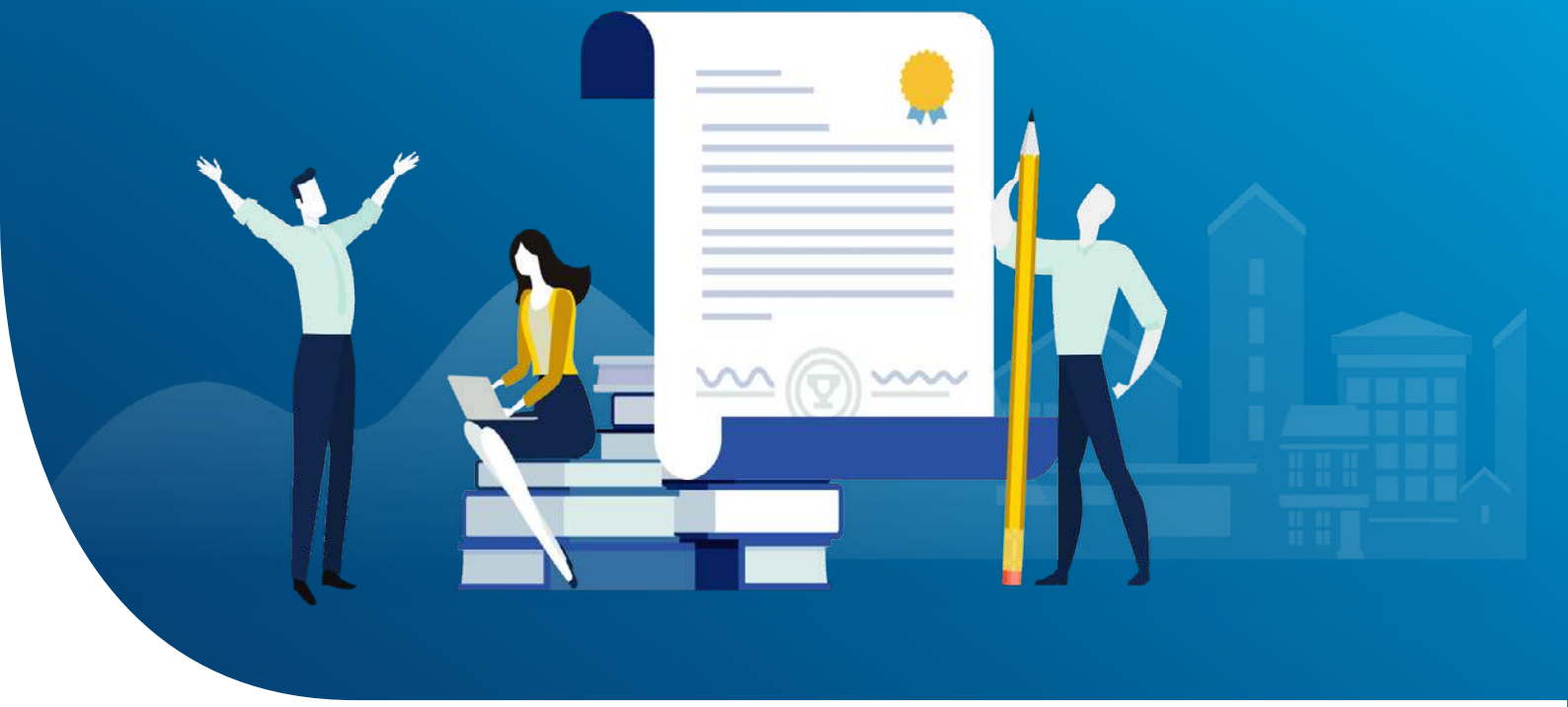
Support for a Well Established Use dossier in the European Union



BlueReg (BR) was involved in the elaboration of a well-established use (WEU) dossier for an orphan drug in a life threatening and debilitating condition for the European Medicines Agency (EMA). BR initiated a systematic review of the literature on clinical aspects to support the WEU justification and updated it in line with regulatory requirements. BR also structured the non-clinical and clinical overview sections of this Common Technical Document (CTD). A regulatory risk / mitigation assessment was conducted for each WEU eligibility criteria in view of an EMA pre submission meeting.

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Challenges

This project consisted in providing regulatory and scientific writing support for a well established use (WEU) dossier for an orphan drug as per the article 10(a) of Directive 2001/83/EC, with the following constraints:

- Very tight timelines to review and finalise the WEU justification, update the literature search and define structures of the non clinical and clinical overview sections of the Common Technical Document (CTD).

- Scientific challenges due to the large number of active substances and the rarity of the condition.

BlueReg support

BlueReg put in place a team of highly experienced consultants with solid expertise in regulatory/scientific documentation to meet the specific project needs within short timelines.

A project manager

- Acted as main contact point with the client.

- Ensured coordination of activities between the client and BR consultants, defined timelines and organised follow-up meetings.

- Adapted the BR resources to meet the tight project timelines by promptly involving two additional scientific writing consultants.

Regulatgory affairs and scientific writing consultants

Regulatory actions :

- Reviewed the WEU justification proposed by the client against regulatory requirements and previous EMA approvals as per the article 10(a) of Directive 2001/83/EC.

- Proposed key messages and justification for missing data.

- Highlighted topics for discussion at the EMA pre submission meeting.

- Conducted a risk/mitigation assessment for each WEU eligibility criteria.

- Performed a regulatory analysis suggesting options and anticipating potential outcomes and impacts.

Scientific Writing actions:

- Analysed the client's literature search methodology and results.

- Conducted a systematic review of the literature on clinical aspects in line with the PRISMA guidelines: definition of a protocol, conduct of the search, screening and selection of publications in line with WEU eligibility criteria, and reporting of identified results.

- Updated the WEU justification in line with regulatory and scientific requirements.

- Provided a detailed structure for the non clinical and clinical overview sections of the CTD, including the identified publications where relevant.

Achievements

- The updated WEU justification was fully compliant with regulatory requirements and previous EMA approvals as per the article 10(a) of Directive 2001/83/EC.
- The systematic literature review conducted on clinical aspects provided a solid evidence based structure for the WEU dossier.
- A detailed structure for the non clinical and clinical overview sections of the CTD was provided, including key messages and justification for missing data.
- Key topics were highlighted for effective discussion at the EMA pre submission meeting.
- The client recognised the high regulatory and scientific expertise of BR consultants and was thankful for the BR team contribution to their WEU dossier.