Amsterdam, 17 March 2021
EMA/114636/2021

Subject: Single-arm studies with historical controls for cancer drug development

The European Medicines Agency (EMA) in collaboration with IQVIA is conducting a study titled: “Single-arm studies with historical controls for cancer drug development”. This letter is to briefly explain the rationale for the study and ask if you are interested to contribute to this study.

Historical controls have been used for a long time in the design and/or analysis of clinical trials but numerous concerns have been raised in the past (Doll and Peto, BMJ, 1980, 280(6206), 44), and they are persisting. The aim of the study is to advance the knowledge about using single-arm studies with historical controls for cancer drug development, including for rare cancers, by developing possible recommendations on how to incorporate the historical controls in analysis with minimum bias and when the use of a single arm study with historical controls might provide sufficient scientific knowledge for different types of regulatory decisions.

The study will use results of simulations based on existing data. The study requires access to existing trial and real-world data that are sufficiently comparable and can be analysed in the same programming environment on IQVIA’s secure servers. The study will explore the impact of different sources for historical controls including commercial or academic trials and real-world data in different situations, like when there may be more limited knowledge about important prognostic factors or about the natural history of the disease.

IQVIA is reaching out to you to get access to data to be used for this study. With this letter we would like to emphasize the importance of this work for EMA and thereby strongly support the request made by IQVIA. We aim to publish the study results in a peer review journal and the authorship will take into account the extent of your contribution to the analysis and revising of results.

Please let us know if you would like to discuss your potential collaboration and any practical aspects further.

Yours sincerely,

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