EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

29 September 2023
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European Ombudsman
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CS 30403
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France

Sent by email only: [redacted]

Subject: Reply of the European Medicines Agency in response to the letter of the European Ombudsman concerning "[t]*he proactive transparency of clinical trial data*" (Case SI/3/2023/MIK)

Dear Ms O'Reilly,

We refer to your letter of 23 June 2023, in which you welcome the decision by the European Medicines Agency (the "Agency" or "EMA") to relaunch its policy on the proactive publication of clinical data for medicinal products for human use ("Policy 0070").[1] In the context of that letter, you also raise three questions concerning the plan and timelines, including the next steps for extending the scope and addressing the backlog of procedures under Policy 0070.

At the outset, it is worth recalling that EMA led the way in the field of transparency on clinical data with the adoption of Policy 0070 by its Management Board in October 2014. Two years later, the Agency was proud to publish the first package of clinical documents through our clinical data portal. Regrettably and due to unforeseen circumstances, the Agency suspended the publication at the end of 2018 due to the activation of its business continuity plan (BCP) linked to Brexit.[2]

In 2020, facing an emergency health threat linked to COVID-19, it became clear that there was an urgent need to share clinical data linked to COVID-19 vaccines and treatments. Consequently, we decided to restart the publication of clinical data exclusively for COVID-19 vaccines and

1 In this respect, see: the "European Medicines Agency policy on publication of clinical data for medicinal products for human use" of 21 March 2019 (EMA/144064/2019); available at: https://www.ema.europa.eu/en/documents/other/european-medicines-agency-policy-publication-clinical-data-medicinal-products-human-use_en.pdf.

2 In this respect, see: Chapter 1 of the EMA Annual Report for 2018 (page 33) which explains that: "*To free up resources to focus on medicine evaluation and supervision, progress in implementing EMA's landmark policy on clinical data publication was halted in August 2018. Pending applications were completed and the publication of clinical data from new applications was suspended thereafter*"; available at: https://www.ema.europa.eu/en/documents/annual-report/2018-annual-report-european-medicines-agency_en.pdf.

treatments going further than regulatory procedures in the scope of Policy 0070 as part of our exceptional transparency measures.[3]

Now that Brexit BCP and COVID-19 BCP have been phased out, the Agency decided to relaunch clinical data publication (also referred to as "CDP") with the support of the Management Board in December 2022.[4]

As our plan to relaunch clinical data publication is evolving, we are happy to address the three questions set out in your inquiry.

1. In relation to the first question

Under the first question, the European Ombudsman (the "Ombudsman") asks the following:

> "What is the current plan and timeline regarding the publication of clinical trial data relating to the backlog of procedures completed during the suspension of Policy 0070 (2018-2023) and other procedures that may not immediately fall within the scope of the relaunched policy? Has EMA consulted stakeholders (both the pharmaceutical industry and civil society) in the development of the current plan and timeline (and, if not, why not)?"

The first question comprises two parts, which are addressed in the following sub-sections.

1.1 Response to the first part of the first question concerning the timeline and plan for clinical data relating to procedures which were not published between 2018-2023 and which do not fall under the scope of the relaunch of Policy 0070

Our current plan was agreed by the Agency's Executive Board in October 2022 and endorsed by the Management Board in December 2022.

It consists of a phased approach to CDP relaunch. This phased approach was carefully considered taking into account the need for all actors involved to make the necessary preparations for restarting activities linked to Policy 0070 and in parallel continuing with the publication of clinical data for COVID-19 vaccines and treatments.

Two options were then considered, either to relaunch fully in one step, meaning a substantial delay to the relaunch, or to start as soon as possible with a more limited scope. Consequently, and taking into account the finding that medicinal products containing a new active substance ("NAS") are of great interest to the public (for the reasons that will be explained under section 2.1 below), it was decided to relaunch the policy as soon as possible in a phased approach, i.e. focussing first on initial marketing authorisation applications ("MAAs") for NAS for which the outcome of the assessment leads to a positive CHMP Opinion, a negative CHMP Opinion or a withdrawal of the application

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[3] The exceptional transparency measures adopted by EMA in response to the COVID-19 pandemic may be found on EMA's website, at: https://www.ema.europa.eu/en/human-regulatory/overview/public-health-threats/coronavirus-disease-covid-19/treatments-vaccines/transparency-exceptional-measures-covid-19-medicines.

[4] In this respect, see: the Highlights of the 118th meeting of the Management Board of 14-15 December 2022, which were published on 16 December 2022; available at: https://www.ema.europa.eu/en/news/ema-management-board-highlights-december-2022-meeting.

as of the date of CHMP plenary meeting in September 2023. In a second step, the scope of CDP relaunch will be broadened to also include extensions of indications and line extensions.

At this point in time of the relaunch, however, it is currently not possible to provide a precise indication as regards to when the backlog of procedures will be published. Between 2018-2023 whilst CDP was suspended, procedures that were initially intended to fall under the scope of Policy 0070 (namely initial MAAs submitted from January 2015 which were non-COVID-19 related) were not published. These so-called "legacy procedures" amount to over 1,600 procedures. In view of the significant volume they represent, it is essential to carefully consider how these legacy procedures will be handled in the future. An overall strategy to manage these legacy procedures is currently under development as part of an ongoing overall review of the current CDP process. This will be subject to consultation with stakeholders prior to finalisation.

It is also important to understand that the proactive publication of clinical data depends, to an overwhelming extent, on the cooperation of marketing authorisation applicants and holders as regards compliance with the policy. Following the decision to suspend Policy 0070, many companies, as well as small and medium-sized enterprises, decided to divest their resources from this activity. In turn, this means that many organisations have been increasingly approaching EMA for assistance in relation to the practical implementation of Policy 0070 in the absence of sufficient in-house knowledge on the preparation of their clinical data submissions.[5] This is a source of additional pressure on the over-stretched resources which are currently assigned for the implementation of Policy 0070.

1.2 The second part of the first question concerning the relaunch of Policy 0070 and the aspect of public consultation

The second part of your first question essentially asks whether EMA consulted the necessary range of stakeholders when drawing up its current plan and timeline for the relaunch of Policy 0070.

EMA would like to confirm that transparency and engagement with the public remains a key principle guiding the implementation of Policy 0070.

As the plan to relaunch CDP evolved, EMA consulted its stakeholders in various formats following the announcement in December 2022 that Policy 0070 would be relaunched. In this respect, EMA would like to refer to the following forums in which the plan was presented and discussed with stakeholders:

- The Drug Information Association ("DIA") Europe conference on 23 March 2023 in Basel.

[5] Marketing authorisation applicants are encouraged to contact EMA for assistance in relation to the implementation of Policy 0070 in writing and also by means of a dedicated preparatory meeting. The latter is a free-of charge service which is provided by the Clinical Data Publication Service. In this respect, see: question and answer 1.16 (page 13) of EMA's "Clinical Data Publication (CDP) Questions and Answers (Q&As) on the External Guidance on the implementation of the European Medicines Agency policy on the publication of clinical data for medicinal products for human use (Policy 0070)" of 26 July 2023 (EMA/14227/2017, Rev. 3); available at: https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/questions-answers-qas-external-guidance-policy-0070-clinical-data-publication-cdp_en.pdf.

- The 10th meeting of the industry stakeholder platform on the operation of the centralised procedure for human medicines which took place on 27 June 2023.[6]
- And, most importantly, a webinar dedicated to the relaunch of Policy 0070 which took place on 16 May 2023.[7]

As regards the webinar of 16 May 2023, it may be highlighted that it was open to all stakeholders who had the opportunity to raise questions and to express their views in response to the proposed relaunch of the policy during the question and answers section. The webinar was recorded and has been published on the EMA website. More than 900 participants registered to attend this webinar and the recording and presentations have been accessed by more than 3000 viewers to date.

In addition, for the purpose of addressing the questions that arose during the webinar of 16 May 2023, EMA informed participants that the answers to the questions raised would be reflected (to the extent relevant) in an updated version of EMA's guidance document relating to the publication of clinical data.[8]

It is also worth noting that after this webinar, a large number of viewers consulted the CDP portal. Indeed, we noticed the highest monthly traffic in May 2023 with more than 3100 viewings of published clinical documents.

In turn, what is important to emphasise is that the participatory nature of the webinar of 16 May 2023 effectively enabled an exchange of information and dialogue between EMA and stakeholders. For completeness, it may also be noted that EMA did not receive any questions or comments which expressed disagreement with the scope of the relaunch.[9]

Based on the above considerations, EMA would like to respectfully submit that the necessary range of stakeholders have been consulted in connection with the relaunch of Policy 0070.

Our decision to opt for a more open form of consultation, as opposed to a formal written consultation procedure, was considered to be proportionate in light of the fact that the Agency is gradually restarting Policy 0070, as opposed to introducing any revision to the policy. Further, EMA also considered that it would be in the interest of the public to ensure the timely delivery of the relaunch of Policy 0070 rather than devoting efforts to a more formal consultation procedure, which could have potentially delayed the timing of the relaunch. Should the policy be updated in the future, EMA will consider undertaking a written formal consultation with all relevant stakeholders.

We will also report on the experience with the relaunch and present plans for the extension in scope, consulting on the proposed strategy for managing the legacy procedures with our key stakeholder

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6 In this respect, see: the presentation relating to the relaunch of Policy 0070, which was delivered, on 27 June 2023, by EMA at the (tenth) meeting of the industry stakeholder platform on the operation of the centralised procedure for human medicines. The presentation of EMA is available at: https://www.ema.europa.eu/en/documents/presentation/presentation-update-policy-0070-k-quigley-ema_en.pdf.

7 In this respect, see: the presentations which were delivered during the webinar of 16 May 2023; available at: https://www.ema.europa.eu/en/events/clinical-data-publication-policy-0070-re-launch-ema-webinar.

8 EMA's "Clinical Data Publication (CDP) Questions and Answers (Q&As) on the External Guidance on the implementation of the European Medicines Agency policy on the publication of clinical data for medicinal products for human use (Policy 0070)", op. cit. updated on 26 July 2023, after the webinar of 16 May 2023.

9 In general, the majority of the questions raised pertained to confirming (without challenging) the precise contours of the scope of the relaunch of Policy 0070.

groups (namely, the Patients' and Consumers' Working Party ("PCWP"), the Healthcare Professionals' Working Party ("HCPWP") and the Industry Standing Group ("ISG").

Further, to the extent that consultation is an iterative process, it is clear that EMA relies on an open and collaborative approach with its stakeholders, particularly in the context of the relaunch of Policy 0070.

2. In relation to the second question

By its second question, the Ombudsman asks the following:

> "Has EMA considered whether the partial relaunch of Policy 0070 could be based on priority areas (such as the publication of data pertaining to the treatment of specific conditions other than COVID-19), rather than on the temporal criterion and the criterion of new active substances? In this context, has EMA considered other criteria and if so, what criteria?"

2.1 Response to the second question concerning the scope of the relaunch of Policy 0070

As a preliminary remark, EMA would like to highlight that the designation of NAS status is granted to medicinal products which transverse a range of therapeutic areas. This includes, but is not limited to the areas of oncology, cardiovascular diseases, diabetes, HIV and AIDS, immune-system diseases, neurodegenerative diseases and viral diseases. It may also be added that medicinal products containing a NAS (as opposed to a known active substance) tend, in principle, to present an element of innovation. Further, several medicinal products containing a NAS are of particular interest in terms of therapeutic value, as they may increase the existing armamentarium of medicinal products.

In addition, a significant number of products authorised as orphan[10] and/or as advanced therapy medicinal products[11] contain a NAS; this entails that these products (that are in principle of public interest) would largely also fall within the initial scope of the relaunch of Policy 0070.

It may also be added that when planning the relaunch of Policy 0070 in two steps, EMA looked at the number of viewings and downloads on the CDP portal and at requests for access to clinical documents under Regulation (EC) No 1049/2001. On the basis of that review, it was found that (unbiased) trends were very difficult to identify in terms of therapeutic area, type of medicinal product or type of regulatory procedure.

It is for the above reasons that EMA decided to focus its efforts and resources –as part of the initial relaunch of Policy 0070– on medicinal products containing a NAS. It is anticipated that the publication of clinical data pertaining to these categories of medicinal products may satisfy the interests of various stakeholders. In particular, this includes the following:

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10 In particular, Article 3(1) of Regulation (EC) No 141/2000 (the Orphan Regulation) sets out the applicable legal framework for obtaining orphan designation, which are intended for the treatment of rare diseases.

11 As recognised by Recital 5 of Regulation (EC) No 1394/2007 (the ATMP Regulation), advanced therapy medicinal products may be characterised by their novel, complex and technically specific particularities.

- **members of the public** who may have an interest in reviewing the clinical data of innovative medicinal products which are intended for the treatment and prevention of very challenging medical conditions;
- **healthcare professionals** and **patients** who may seek to understand the therapeutic landscape relating to new treatments, in particular, in the scenario whereby a product is intended for an unmet need;
- **researchers**, **academia** and **sponsors** of clinical trials who may wish to learn from the success or failure of clinical development programmes; and
- **health technology assessment authorities and bodies** who may seek to understand the clinical data relating to treatments which have an increased effectiveness or milder safety profiles compared to existing treatments.

It is also important to note that the decision to focus on medicinal products containing a NAS as part of the initial relaunch of Policy 0070 in no way presupposes that EMA has definitively excluded the possibility that products containing a known active substance will be subject to Policy 0070 in the future.

3. In relation to the third question

By its third question, the Ombudsman asks the following:

> "What is the current timeline and plan for the next phases of expanding Policy 0070?"

3.1 Response to the third question

As stated above, the scope of step 1 of the CDP relaunch is to publish, in addition to COVID-19 medicinal products, clinical data submitted as part of initial MAAs for NAS for which the outcome of the assessment results in a positive CHMP Opinion, a negative CHMP Opinion or a withdrawal of the application from the time of CHMP plenary meeting of September 2023. We currently anticipate that approximately 20 to 25 regulatory procedures between September and December 2023 will be subject to step 1.

In order to build further capacity and improve efficiency, the CDP Service is currently working on improving processes, technical tools and documentation such as a revised template of the anonymisation report in view of preparing for step 2 of the relaunch of Policy 0070, also with the support of an external reputable service provider. In addition, we are collaborating on an international level with Health Canada, who has implemented a very similar approach to CDP, to share experience and promote work sharing as far as possible. A strategy for the management of legacy procedures is under development and, as highlighted above, will be subject to consultation with stakeholders prior to finalisation.

Further to the above, it is not possible to provide an exact indication as regards the timeline for implementing the next phase (step) of the relaunch of Policy 0070. At this stage, however, we currently anticipate that step 2 of the relaunch will move beyond NAS-containing medicinal products to regulatory procedures involving extension of indication applications and line extension applications in 2024.

We hope that the clarifications provided demonstrate the endless efforts that EMA makes in delivering a high level of transparency in the field of clinical data, which is aligned with EMA's values and with our long-standing cooperation with the European Ombudsman in this field.

We remain available to provide any further information that could be useful for the purpose of this inquiry. If you believe that a meeting would be necessary to discuss any of the foregoing, we would be happy to organise it at your convenience.

Yours sincerely,

[redacted]
Executive Director