ECL's Access to Medicines Task Force and EORTC welcome CJEU ruling on the EMA's transparency policy

22 January 2020, Brussels

The Access to Medicines Task Force of the Association of European Cancer Leagues (ECL) and the European Organisation for Research and Treatment of Cancer (EORTC) strongly welcome today's Judgment[1] by the Court of Justice of the European Union (CJEU) which confirmed the right of access to documents related to the marketing authorisation application of medicinal products. Clinical study reports have been publicly accessible since 2010, and starting from 2016, the European Medicines Agency (EMA) has been systematically publishing these reports on its website.

EMA's clinical study reports include comprehensive information on the design, methods, analyses and results of clinical trials. Access to such reports provides the opportunity for independent research, assessment of reporting and evaluation of bias, detailed evaluations of related harms and adverse events, trial re-analyses and their integration in systematic reviews and meta-analyses.

An open letter[2] supported by more than 30 health organisations stated that disclosure of clinical data and information, including clinical study reports, is of vital importance for patients and for healthcare professionals, researchers, HTA bodies, independent drug bulletins, healthcare payers, the global health community and the general public.

Two drug companies challenged the EMA's right to grant external access to clinical study reports for a toxicity study of MSD Animal Health and a clinical study report for PTC Therapeutics. The General Court of the EU dismissed both actions in 2018,[3] but the companies appealed against the judgment before the CJEU. The CJEU did not follow the opinion of Advocate General Gerald Hogan delivered on 11 September 2019,[4] in which he considered that the disclosure of clinical study reports might undermine companies' commercial interests. This could have compelled the EMA to revise its transparency policies and close access to information submitted in the context of the procedure relating to medicines marketing authorisations. ECL and EORTC are delighted that the final rulings focus primarily on the benefits of transparency on patient safety, rather than commercial interests.

The importance of transparency for patients is exemplified by a trial case published in The New England Journal of Medicine in 2017.[5] In this trial, patients newly diagnosed with metastatic castration-sensitive prostate cancer (mCRPC) were randomly assigned to receive androgen-

[1] Judgement of the Court of 22 January 2020, PTC Therapeutics International Ltd v European Medicines Agency Case (C-175/18 P), ECLI:EU:C:2020:23

[2] EPHA (2019) Joint open letter on access to clinical study reports, Available at: https://epha.org/protect-open-access-to-clinical-study-reports/

[3] Judgments of the General Court of 5 February 2018, PTC Therapeutics International v EMA (T-718/15) and MSD Animal Health Innovation and Intervet international v EMA (T-729/15)

[4] Opinion of Advocate General Hogan of 11 September 2019 (Joined Cases C-650/17 and C-114/18)

[5] Fizazi, K. et al. (2017) "Abiraterone plus Prednisone in Metastatic, Castration-Sensitive Prostate Cancer". The New England Journal of Medicine, 377, 352-360. Available at: https://www.nejm.org/doi/full/10.1056/NEJMoa1704174

deprivation therapy plus placebo or abiraterone and prednisone. The trial results showed a significantly better overall survival and progression free survival in the experimental group. During the trial, Abiraterone was approved for mCRPC and the trial seemed to confirm that Abiraterone should also be used in patients where the prostate cancer is still castration-sensitive. Subsequently, Abiraterone was approved by EMA for this indication.

Upon closer scrutiny, the trial results raise some questions. The trial was conducted mostly in countries where Abiraterone was not available. In these countries, Abiraterone was not the standard of care in patients with mCRPC. Consequently, only a minority of patients in the placebo group received the standard of care when they became castration resistant. When comparing overall survival in the experimental group and the control group, the added value of Abiraterone may have been exaggerated. Therefore, findings of the trial should be interpreted with caution when adjusting clinical guidelines. In addition, in the trial the dose of prednisone to counteract the side effects of Abiraterone was reduced, which resulted in a strong increase of side effects. The rationale for that change in prednisone dosage has never been explained and justified in the publication.

Such issues could have been avoided through early dialogues and engagement of academics and patients in the trial design. Moreover, it is important that independent researchers can scrutinise and analyse the trial data, results and designs after the marketing authorisation has been approved. In addition, access to these reports helps researchers and healthcare professionals to develop guidelines that are scientifically correct and relevant for patients.

The fact that only a minority of patients in the placebo received Abiraterone, for instance, was not clear in the published article. Therefore, it is vital for the health community to be able to have access to the detailed information included in the EMA's clinical study reports and thus today's ruling was so crucial.

Finally, in addition to the most-welcome practice of allowing access to clinical study reports, the ECL and the EORTC encourages the EMA to go a step further in their transparency journey. We would be pleased to see a more robust scrutiny of clinical trials, including requesting the full dataset of raw data as the USA's Food Drug Administration (FDA) does routinely and allowing for an independent analysis of such datasets. This, we believe, would reassure the wider health community about the added value and safety of the medicinal products authorised in the EU.

About ECL's Access to Medicines Task Force

The Association of European Cancer Leagues (ECL) is a non-profit, European umbrella organisation of national and regional cancer societies. Since 1980, ECL provides an exclusive platform for members to collaborate with their international peers, primarily in the areas of cancer prevention, tobacco control, cancer research, access to treatments and patient support services, and creates opportunities to advocate for these issues at the EU level.

Established in 2016, the ECL Access to Medicines Task Force advocates for equal access to cancer treatments for all patients in Europe. The Task Force's goals, defined in the Declaration of Intent, are to reach access to high value innovative medicines, transparent and fair pricing of medicinal products, improved patient participation and sustainability of healthcare systems. The Task Force strongly believes in the power of dialogue. We urge all decision-makers and stakeholders to work together and push for equal access to high quality treatments improving both survival and the quality of life of cancer patients.

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About EORTC

Founded in 1962, the European Organisation for Research and Treatment of Cancer (EORTC) is an independent, non-profit cancer research organisation, with a mission to coordinate and conduct international translational and clinical research to improve the standard of cancer treatment for patients. Our work spans tumour types, disciplines and national borders, comprising a network of over 930 institutions in 30 countries. We ultimately aim to increase people's survival and quality of life by testing new therapeutic strategies based on existing drugs, surgery and radiotherapy. We also help develop new drugs and approaches in partnership with the pharmaceutical industry and in patients' best interests.

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