MEETING MINUTES
10th CML Community Advisory Board (CML-CAB) / 20th CAB CML-session
Incyte/Takeda – 23 July 2020, 17:30-20:30 CET

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Executive Summary

The CML Community Advisory Board (CML-CAB) is a workgroup of the CML Advocates Network operating since 2016 and focusing on the harmonization of good clinical practice, standards of care and access to best available CML therapies and diagnostic and monitoring tools. CAB meetings provide an opportunity to build a meaningful dialogue between pharmaceutical companies and the patient community to overcome the challenges and needs associated with chronic myeloid leukaemia (CML). The main objectives of this first virtual CML-CAB meeting with focus on "global access" were to foster a mutual understanding and to take tangible actions forward for Incyte (who commercializes ponatinib, a third-generation tyrosine kinase inhibitor (TKI) in European Union plus additional countries) and Takeda (who commercializes it in the United States and Australia), the patient community and both in collaboration. Topics discussed during the meeting included the results of a survey on access to ponatinib conducted by CML Advocates Network in the period March to June 2020, the current patient access status in EU/Europe, Africa, LATAM & Asia, an update on the OPTIC study & the PACE study to address patient community questions about ponatinib safety, dosing, and side effect management, as well as access to mutation testing & mBCR ABL monitoring. Sessions were followed by discussion rounds, enabling the sharing of different perspectives and offering both sides the chance to ask questions.

The meeting started with a formal opening and a quick introduction round of all 33 participants (16 CML-CAB members, 7 Takeda representatives, 7 Incyte representatives, 2 members of the CML-CAB Management team, and the professional minute writer). Following that, the **"Progress Card"** was presented as a future tool to evaluate progress made in-between CML-CAB meetings. Parties agreed that the "Progress Card" should be a solution-oriented tool to track progress of shared priorities and to acknowledge challenges in connection with the global access situation of ponatinib. Companies expressed that they wished to have one rating for both companies since they aim at working collaboratively towards solutions.

The next session was dedicated to **"Access to Ponatinib"** and started with a presentation of the results of a survey which was conducted by CML Advocates Network in the period March to July 2020 and consisted of 9 questions covering access to ponatinib, access to mutation testing, relationship with distributor and information needs of patients. The survey was answered by patient representatives from 41 countries/regions and revealed the following main findings:

1. Major access issues in some countries of Eastern Europe, Africa, LATAM & Asia (though access on a humanitarian basis is possible through The Max Foundation in 59 countries).
2. The community's perception is that the main *single* access barrier is absence of registration for ponatinib in > 15 countries across all geographical regions, specifically Algeria, Armenia, Costa Rica, Central America (Costa Rica, Guatemala, Nicaragua, Honduras, El Salvador, Belize, Panama), Georgia, Indonesia, Kazakhstan, Macedonia, Madagascar, Morocco, Philippines, Russia, Serbia, Ukraine – as per the survey. 42% of responses across all geographical regions show that reimbursement/co-pay is the *main* overall issue.

3. Access to mutation testing is an issue in many countries with barriers to testing divided between lack of physician education, payor coverage and availability of technology
4. Side-effect profile of ponatinib also a concern.
5. Main patient information needs revolve around side-effect and co-morbidities management prior and during treatment as well as and education on mutation testing.

To track requests and follow-up on specific critical access cases, it was agreed that individual patient access issues that might be identified by any member organization will continue to be channeled through the Executive Director of the CML Advocates Network and the company team leads. Takeda and Incyte both committed to connect select CML-CAB members with company representatives or distributors in their specific country or region, and to confirm Patient Services Programs as well as current access channel in select countries.

The presentation of the **OPTIC and PACE study** results lead to a series of questions by the CML-CAB members. Following these, the study of ponatinib in combination with other therapies (especially for resistant or intolerant patients) was discussed, and it was agreed to mutually explore opportunities in the wider life-sciences community to determine its feasibility. It was further agreed that companies would look into sub-populations that reflect real world patient scenarios.

Discussion on access to mutation testing & practice of testing (specifically mBCR ABL monitoring) confirmed the need to enhance efforts to improve access/education/innovation on that topic. Following the presentation by Incyte of Think Test Treat campaign, a programme initiated by Incyte to raise awareness of the importance of regular and sensitive monitoring and mutation testing in patients with Ph+ leukaemias, Incyte expressed the desire to work with the CML Advocates Network to evolve the initiative together. There is a strong wish to explore opportunities and start defining a roadmap with piloting countries, potentially in the framework of a workgroup including the CML Advocates Network and Takeda / Incyte.

All parties expressed their clear commitment to continue the discussion, follow up with concrete actions and work towards tangible solutions and ultimately improve the access situation and information needs of CML patients worldwide.

Meeting moderated by:
Jan Geissler & Pat Garcia-Gonzalez (CML-CAB chairs)

Minutes prepared by:
Denis Costello (Executive Director CML Advocates Network) and Nicole Schröter (CML-CAB officer)