EXECUTIVE SUMMARY of the 22nd CML Community Advisory Board Session (CML-CAB) with Novartis – 18 February 2021, 16:30-19:30 CET

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CML Community Advisory Board (CML-CAB) is a working group of the CML Advocates Network operating since 2016. It is a global panel of 19 leading patient advocates from all world regions ("CML-CAB members") who all represent the unique perspective of a CML patient or relative. CML-CAB members work together to address issues of strategic importance to the community and advocate for the best possible research and equal access to the most innovative treatment & care for CML patients around the world. CML-CAB monitors pharmaceutical developments and research in CML through active and targeted interaction, long-term cooperation and continued dialogue with pharmaceutical partners, regulators, medical experts, and the scientific CML community.

The main objectives of this 22nd CML-CAB session (9th with Novartis) which took place in a virtual format and whose focus was on "Future Treatments and Current Access" were to foster a mutual understanding and to take tangible actions forward for Novartis, the patient community and both in collaboration. Main focus of the meeting was asciminib (ABL001) – Novartis´ novel investigational treatment that specifically targets the ABL myristoyl pocket (STAMP). Topics discussed during the meeting included the Asciminib Development Program (incl. Patient Reported Outcomes measures and Quality of Life), its value proposition and positioning of the drug, as well as Novartis´ global access strategy for asciminib.

A multi-centennial body of experience consisting of 13 CML-CAB members and covering 241 patient or advocacy years attended this meeting. Novartis was represented by the senior level experts in Global Patient Engagement, Medical Affairs, Drug Development, and Global Value and Access. CML-CAB members selected to attend this specific meeting were chosen out of the total group of 19 CML-CAB members (altogether relying on nearly 340 patient or advocacy years), based on expertise on the meeting topics. Since the CML community has sadly lost 4 valued and engaged advocates in the past 8 weeks only, in a moving speech Denis Costello, Executive Director of the CML Advocates Network and meeting moderator, encouraged the participants to take a moment of silence in remembrance of those who have gone before us, and "to remember why we are here and who we serve".

Following that, two senior CML-CAB members presented the **"Progress Card"** - a tool that reflects CML-CAB´s strategic priorities (Clinical/Research, Access, Collaboration & others) and is routinely used by CML-CAB to evaluate progress made in-between CML-CAB meetings. While the "Progress Card" acknowledged progress made in certain areas, it also highlighted areas in which the community's expectations were not met or where CML-CAB´s advice had - from a CML-CAB perspective - not been acted upon satisfactorily. For reasons of time, Novartis was not asked to comment on the "Progress Card''.

In the next session, four advocacy experts that are part of the **"Access Working Group"** presented the CML-CAB´s report on and evaluation of this distinct working group that – as a result of the 2019 CML-CAB with Novartis in mid-2020 – was established as a pilot-project. The "Access Working Group" consists of a fixed group of people and aims at tackling access issues in 5 specific, previously selected countries where access to imatinib and nilotinib remains to be an unresolved issue for underserved populations, and where the community feels Novartis can play a larger role to help patients, i.e. Philippines, Kosovo, Mexico, Venezuela, and Bangladesh. Working Group members on both sides (Novartis and CML-CAB)

meet at regular intervals to 1. Align realities and expectations, 2. Agree on the problem/unmet need, 3. Explore solutions on how to solve the unmet need and enable access to these lifesaving drugs to CML-patients in the above listed countries. The community noted that since July Novartis has provided feedback for only three countries: Philippines, Kosovo and Mexico. The leading advocate from each of the three countries presented a summary of progress. The assessment from the CAB is that while the discussions that have been had are productive, Novartis has been slow to provide feedback and answers. Novartis committed to move things forward by setting-up follow-up meetings in Q2 2021.

The next session was dedicated to **"Asciminib development program: Past, Present and Future"**. Since the presentation had been shared with CML-CAB members in advance to the meeting, Novartis´ representatives from Medical Affairs and Global Development highlighted just some key facts, enabling the group to use the rest of the time for an engaged discussion. . While CML-CAB acknowledged that with Asciminib the community has been involved more than in any previous drug development, there was still dissatisfaction about the fact that – up until now – Novartis is not following the community´s advice to give preference to specific PRO-tools. Since January 2017 the community has been calling on Novartis to use the EORTC-QLQ30+CML24 instead of MDASI, because it more sensitively reports about CML-specific symptoms, side effects and anxiety. Novartis confirmed that all new asciminib trials initiated by the Novartis Global Drug Development team include the EORTC QLQ-C30 plus CML24 recommended by the community. Furthermore, the community has repeatedly asked Novartis to provide data on toxicity measurement over time. CML-CAB members expressed disappointment at the lack of these data being reported at this CML-CAB, especially since the community has no insight into how Novartis is measuring toxicity and the development of adverse events (AEs) in its studies. Novartis reiterated the standard process of AE collection in the Novartis studies and that the existing toxicity profile data from the Phase 3 asciminib trial (ASCEMBL) was shared with the community. Additional safety data analysis is ongoing. What was furthermore challenged was Novartis´ decision to not add new countries and study sites. Novartis explained the complexities of the site allocation process and why sometimes they need to make difficult decisions about limiting a number of participating countries to streamline operations. The Company made a commitment to further explore opportunities of expanding the footprint for the studies and look into the countries that have not been previously considered as candidates. Last but not least, CML-CAB again reminded Novartis that CAB is operating under confidentiality and requested Novartis to please share data even before it is in the public domain and discussed with regulators. In a passionate statement a CML-CAB member urged the company to involve the community early and in a timely manner, not only to help CML-CAB really understand the benefits of the drug compared to currently available TKIs (e.g. with regards to long-term side effects and toxicity) but also to help the company define and communicate its value story, given the community largely expects issues with reimbursement bodies given the current approach to data reporting seems to focus mainly on clinical efficacy and not solid evidence of tolerability and AEs.

This session was followed by one entitled **"Asciminib – Value proposition & positioning: Novartis´ approach and community view"**. Value proposition is generally defined as a promise of value to be delivered, communicated, and acknowledged. In the pharmaceutical industry, value proposition consists of two core elements: price on the one hand; clinical benefit on the other[1]. A Novartis colleague from Global Value and Access presented a high-level outline of what Novartis is currently testing with payors, while highlighting that separate message testing was being done with other stakeholders (patients,

[1] https://www.pharmexec.com/view/continuity-and-decline-of-the-pharma-value-proposition

HCPs, etc.). She started her talk by explaining why – from a Novartis perspective and specifically to the "3rd line plus setting" – there is substantial unmet need for patients, and how the company hopes that asciminib as the first-in-class STAMP inhibitor will address these challenges. In terms of process, she described that at the end of a value proposition process, a dossier is created which highlights the data coming from clinical trials (while real world evidence is also being collected), cost effectiveness analysis, as well as budget impact models to be submitted to the payors / healthcare systems. Again, an engaged discussion followed the presentation. The community expressed disappointment with regards to Novartis not clarifying the value proposition intention in earlier lines. Novartis highlighted that the value proposition was still work in progress and not set in stone, which led CML-CAB representatives to urge Novartis to be involved in this process, especially when it comes to aspects on quality of life and AEs since this is what the global community of CML patients is strongest and feels most empowered to support. Last but not least, a CML-CAB representative reminded Novartis of the fact that patient advocates are "here to advocate for access (…). Let us help, let us discuss the draft stage. We are not here just to comment and critique, we're here because we're trying to secure something for our patient community". It was suggested to further elaborate on this within the framework of the "Asciminib Working Group" (or a subgroup of the same), a working group actively operating since 2019 and composed of ten top-level CML-advocates.

The last session was on **"Asciminib – global access strategy, access in non-commercial countries and role of molecular monitoring"**. Novartis shared a high-level overview of the global access strategy principles for asciminib without providing any details on specific access approaches since this information is exclusive to a very limited number of people within the company due to its commercially sensitive nature. The community, however, clarified that it is asking for a broad strategy, not for an individual list of countries, and that it would already be a satisfying first step to know that there is actually an access strategy with global reach.

Generally speaking, Novartis accepted that the timing of sharing certain information could be improved, and committed to exploring further as to which information exactly can and should be shared at early stages.

The CML-CAB co-chair concluded the discussion by strongly suggesting to everybody at Novartis involved with asciminib and CML "to actually look at the situation of the global CML-community" since CML is very different to other diseases. "There are CML-patients that we know personally in every single corner of the world, and because the treatment is long term we've known some of these people for 20 years." Novartis should not miss the opportunity to hear them through CML-CAB who is giving them a voice.

The moderator thanked the Novartis participants for their time and insight, and also for the commitments made in the course of the meeting. The group will follow up with concrete actions and work towards tangible solutions and continue the dialogue.

Meeting moderated by:
Denis Costello (Executive Director CML Advocates Network)

Minutes prepared by:
Nicole Schröter (CML-CAB officer)