

MEETING MINUTES

19th CML Community Advisory Board (CML-CAB) (9th with Novartis) on November 20, 2019

Executive Summary

CAB meetings provide an opportunity to build a meaningful relationship between pharmaceutical companies and the patient community to overcome the challenges and needs associated with CML.

Topics discussed during this 9th CML Community Advisory Board (CML-CAB) meeting with Novartis included goals, issues and expectations from the CML community's perspective, a discussion on patient-centric cure for CML and Q&A sessions on access today and tomorrow and on CML patients' inputs into R&D.

Discussions focused mainly on how to collaborate best to tackle issues with access to treatment/monitoring and clinical trials in LMICs and how to move forward towards a patient-centric cure for CML with CML-CAB.

CML-CAB will define their access priorities and two (2) Working Groups on access will be set up on 1) Access priorities & challenges (for a broader view on access challenges) and 2) Access planning: Data & Asciminib value proposition. These two Working Groups will be added to the existing one established in October 2019 on the Asciminib development programme.

There was a strong call from the patient community to share the patient engagement roadmap for Asciminib and to involve the CML patient community early and systematically into the clinical development program.

Both sides agreed on creating a mutual partnership between NVS and CML-CAB with a continuous dialogue in between CAB meetings. It was discussed to open CAB meetings also to other groups (e.g. academia, diagnostic companies) and to organize multi-stakeholder meetings.

All parties agreed that CAB Meetings are an important forum and expressed their clear commitment to meet again and continue discussions to foster understanding of shared issues and find solutions.

Topic 1: Goals, issues and expectations from the CML-community's perspective

CML-CAB members from Asia, Africa, Europe, Latin America and North America shared the reality of living with CML in their regions and discussed specific challenges and expectations from the regional CML community's perspectives.

Key points from the discussion:

- Experiences of patients in the Western world are different from the experiences of patients in low- and middle-income countries (LMICs).
- In particular, access to treatment, monitoring and clinical trials should be improved.
- In the GIPAP program with 85,000 patients, a medium age of 36 at diagnosis (including India) was reported, in contrast to the official median age of 55.

- Treatment discontinuation (and the concept of treatment-free remission, TFR) does not play a major role outside of the Western World due to limited access to monitoring in LMICs
- Main CML community issues identified are: inequities of access to TKI's and monitoring, a disconnect between guidelines and clinical reality, an increasing lack of interest in pursuing a cure for CML, issues associated with long-term TKI use, lack of clinical trials, long distance travel to treatment centre, stigma and discrimination, political instability, generics, counterfeit products, challenge for PAG's to build capacity and lack of interest to support CML programs.

Topic 2: Moving towards a patient-centric cure for CML

The group discussed what cure means for NVS and for the CML patient community and how they could work together to achieve a patient-centric cure.

Key points from the discussion:

- From the patient community's perspective a cure means to lead a normal life without drugs, side effects and monitoring with the same QoL as other people (currently continuous monitoring is still necessary on TFR which is a constant reminder of the disease and the stigma of cancer adheres to the patients).
- TFR is currently in the focus of scientific and clinical research, but the group agreed that there is a need to look into a real cure addressing the root cause of CML (e.g. via gene therapy). TFR is not considered as a real cure for CML and is not the solution in many LMIC's with limited access to PCR.
- CML-CAB asked NVS to engage here and to continuously evolve research towards a cure for CML. The patient community called out for more collaboration and more funding for research on a cure for CML.
- NVS stressed that they are already working on gene therapies in other disease areas, but that the science is very complex and not yet there in CML. The company stressed the importance of bringing world-renowned scientists to the table to work on a real cure.
- The group agreed on the need to change the narrative here (through PR etc.) and to re-engage companies/academics to search for a cure for CML.

Topic 3: Access today and tomorrow

CML-CAB raised the question what else can NVS/the CML patient community do to solve the remaining access challenges today – predominately, but not only in LMICs | (also US etc.).

Key points from the discussion:

- Given the broad issues NVS asked for input from CML-CAB and to decide on access priorities. The group agreed to set up two (2) different working groups on access:
 - 1) Access priorities & challenges
 - 2) Access planning for Asciminib
 These two Working Groups will be added to the existing one established in October 2019 on the Asciminib development programme.
- The group discussed the idea of a multi-stakeholder meeting on access to facilitate collaboration.

- NVS promised to liaise regional CML-CAB members with NVS regional access teams to facilitate a dialogue on local level.
- Novartis/CML-CAB members agreed to collaborate on generating solid patient preference data.

Topic 4: CML patients' input into R&D

The patient community discussed opportunities to involve the CML patient community early and systematically into the clinical development program using a patient engagement roadmap.

Key points from the discussion:

- Patient engagement roadmaps are now widely-used but there is still a gap between theory and practice from the CML community's point of view
- CML-CAB stressed that the patient community's perception on site selection is completely different than that of the company's perspective. NVS stressed that they made a commitment to *not* choose countries without consulting CML-CAB.
- With view to clinical trials in Africa, NVS reported a pilot project in Ghana. The company currently investigates, if this strategy could also apply to other African countries, but cannot make any commitments now.
- NVS reported that they also have a paediatric development plan in place pushing for a more systematic inclusion of the teenager population, which was appreciated by the CML patient community.

Topic 5: Evolution of CAB meeting

CML-CAB reported that the CAB model was first adopted from the HIV patient community. Since the first CML-CAB meeting there was a learning curve on how to best use these during the CAB meetings. Now other patient communities are following the same model.

Key points from the discussion:

- CML-CAB acknowledged NVS as '*The CML Company*' having pioneered CML. It was also stressed that NVS and the CML patient community have been working together for 20 years.
- CML-CAB members asked NVS to consider the CML patient community as partners and CML-CAB members to provide feedback more proactively.
- CML-CAB members announced that they would explore opportunities for an 'academic CML-CAB' with key opinion leaders, regulators, policy makers and potential other stakeholders to allow a broader stakeholder discussion and to open CAB-Meetings to diagnostic companies/laboratories.