

Sobi Charter on Patient Access Bridging Programmes

Responsible Department: External Affairs & Patient Access

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General

Sobi is committed to playing an active role in the dialogue with stakeholders, governments and healthcare systems across the globe, to ensure that patients get timely and sustainable access to required medicines. We are also committed to providing bridging solutions for patients while these dialogues are active and on-going, provided commitments to building sustainable, long-term solutions are in process.

Sobi develops and provides treatments for several different diseases and conditions, mostly rare, genetic disorders. These may occur in any part of the world, irrespective of the level of development of local healthcare system. This does not mean that the patient community there is any less deserving or any less in need of the treatment than patients in countries or regions with more developed healthcare systems.

Sobi receives requests from patients, patient groups or physicians to receive our treatments, where the local government and/or healthcare system is not – or not yet – including them systematically in the local healthcare provisions. Sobi should be part of finding a way to secure that patients get access to the treatments in a sustainable way, without taking over the responsibility of the payer or other parts of the healthcare systems.

Sobi strives to put the patient need at the forefront of our daily work and in our overall approach to our business. We undertake this commitment in the context of our responsibilities to a broad set of stakeholders – including current and future patients, investors, governments, healthcare systems and employees. None of our stakeholders are well-served if Sobi does not take a responsible and sustainable approach to our business.

1. In order to ensure that we are able operate over the long-term, our first commitment as a company is to ensure that we have secured a sustainable future for all our stakeholders – both internal and external. This includes meeting our financial commitments as a viable company, and to secure a long-term investor base and reinvest in future research.
2. At the same time, we must also strive to acknowledge the fact that *“patients suffering from rare conditions should be entitled to the same quality of treatment as other patients”*. This means that we and local healthcare systems recognise that rare disease patients – while rare – should not be treated as exceptions or aberrations, but an integral part of the healthcare system. This can only be achieved by a concerted, multi-stakeholder dialogue and engagement, with an objective of finding shared solutions on a sustainable basis.

In between these two situations, there may be times where a patient or a group of patients are in need, and where the urgency to ensure availability of treatment means that we should not wait for the successful outcome of this dialog before providing availability to treatment.

Aim of the Sobi Charter on Patient Access Bridging Programmes

To date, Sobi has responded in many instances to provide treatment free-of-charge to patients in different geographies. This has increased in recent years and could lead to situations of inequity or lack of clarity about how we should respond as a responsible company, in order to ensure a sustainable future for our stakeholders.

This makes it difficult to enter into agreements, because it may be unethical to withdraw treatment from a patient, especially where it is life-saving. On the other hand, Sobi is not financially or logistically able to supply free product across the globe on an indefinite basis.

As Sobi expands both in geographical reach and in terms of the therapeutic areas we serve, a more systematic approach is required.

This document is intended to guide Sobi and its partners in the healthcare systems across the globe to understand how we should act under such circumstances and what steps we will take together to achieve a sustainable, shared long-term future for the patient communities that we serve. It also lays out the roles and responsibilities of the different parties.

This Charter may be used both internally and externally as a basis for conversations with patients and patient groups, physicians, governments and government authorities in a given local situation, with the objective of building a transparent shared understanding; and to facilitate an agreed vision and pathway forward in each local situation.

Sobi also acknowledges that we cannot provide solutions for the healthcare needs of patients across the globe without the commitment and engagement of all stakeholders in the local context. Each stakeholder has a role to play. This document is intended to lay these out clearly and to provide the framework for a transparent and documented, mutually agreed set of commitments going forward.

Guiding Principles on the Sobi Charter on Patient Access Bridging Programmes

- Sobi will consider all requests to establish a Patient Access Bridging Programme.
- Sobi will comply with all relevant national guidelines or regulations.
- Rare disease patients should be integrated into the healthcare system as any other patient, irrespective of the prevalence of the disease or condition. Given the serious, chronic and often life-threatening nature of rare, genetic diseases, there is an imperative to secure treatment for such rare disease patients on an immediate but also a sustained basis.
- Integrating patients and their families into healthcare systems implies a variety of different roles, responsibilities and expectations from different stakeholders. All have a role to play and all of them must be fully committed to working together to find a sustainable solution. This includes Sobi, but Sobi cannot do this alone.
- When a Patient Access Bridging Programme is established, it will be as a temporary solution – acknowledging that timelines will differ on each individual basis. It will be based on the willingness of the different actors within a given healthcare system, region or geography to engage in the process. This will be agreed and documented up front, including a shared responsibility for the expected timelines and next steps. This will be reviewed on a regular basis at agreed times.

- Physicians are often the first point of contact for the diagnosis or identification of patient in need of a treatment provided by Sobi.
 - It is vital – for the good of all patients– that Sobi products and treatments are used appropriately, according to the labelled indication. This ensures the best patient outcomes, but also ensures the integrity of the company and the integrity of the product, which has been scientifically evaluated on the basis of its quality, safety and efficacy when used according to the label. Failure to use Sobi’s treatments according to the label may put patients at risk, may raise false hopes and, in some circumstances, may undermine the credibility of the established scientific review and approval systems that have evaluated the treatments.
 - Our connection with the diagnosing and treating physician must respect his or her responsibility and commitment to the patient, while at the same time helping them to understand the commitment that is required to secure a long-term and sustainable treatment for the patient or patients in question.
- Patients or patient groups are in the front-line of the question about sustainable availability of treatments. In many cases this will mean a child, a sibling, a parent or another close relative.
 - We must always be cognisant and respectful of the personal story behind each clinical case. At the same time, we must also work with patients, patient representatives and patient groups to help them understand the importance of engaging with their treating physician to secure an effective dialogue with the local healthcare system.
 - No-one else can explain the impact of a condition on a patient or on a family more appropriately than the patient or the family of the patient. *“Nothing about us without us”* is a patient group statement that can serve all stakeholders well when we consider the best decisions for the patients in a given situation. In other words affected patients and patient organizations should be an active part of any dialog regarding sustainable access to medicines.
 - It will be important for all of us to understand that the patient, patient representatives or a patient group is willing to work with the treating physician to build awareness within the local healthcare system and healthcare providers about the importance of having sustainable access to safe and effective treatments.
- Governments across the world are responsible for providing sustainable healthcare systems, tailored to local needs and conditions. Patients suffering from rare diseases or conditions might not be as “visible” to the governments as those suffering from more common or more well-known conditions. It is vital that the governments are made aware of the burden of disease and the consequences of not treating rare diseases, on society, the families and communities as well as the patients themselves.
 - Funding and structures might already be in place in a given region or geography, allowing the treatment for rare diseases to be integrated into the healthcare system.
 - In other areas, funding might be available via government or agency funding, or via such organisations as the World Health Organization (WHO).
 - The provision of bridging access solutions by Sobi should not be seen as a replacement for an engaged dialogue, with the objective of securing a long-term, sustainable engagement of the products / treatments in the healthcare systems. In general Sobi requires a tangible commitment from the local healthcare system in order to initiate a patient access bridging programme.

- Sobi acknowledges that conversations between all the relevant stakeholders take time. These timeframes might be estimable at the beginning of the process and should be documented as such. However, they might also change. Sobi remains committed to the process as long as the other parties in the process are also committed. While at the same time, acknowledging that providing long-term, sustainable healthcare solutions for patients is not something that can be solely delivered by Sobi on its own. Regular progress reporting / check in points will be a key feature of any agreed Patient Access Bridging Programme.

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