FONDAZIONE TELETHON

Free Access to Treatments Policy

Pre- and post-regulatory approval of access to treatments

1. Who We Are and Why This Policy Exists

Fondazione Telethon was born from the love and determination of families who had lived with rare genetic diseases first-hand: parents, siblings, children who came together to do what health systems alone could not. That same energy, over time, led Fondazione Telethon down an unexpected path - not out of ambition, but out of responsibility.

Fondazione Telethon was created to support and conduct research. Yet, in some cases, ensuring that lifesaving discoveries actually reached patients required us to go further. This is how we became one of the first non-profit organizations not only to fund research, but also to take responsibility for bringing therapies to patients when no other route existed.

This request did not change our **mission**: **we are here to do research and transform it into treatments.** It is not within our mission to subsidize healthcare systems, neither in Italy nor in other countries.

For this reason, Fondazione Telethon has defined a clear and responsible **pro-bono access pathway**, used only as a last resort. Free access may be considered only after all other possible options have been genuinely exhausted.

The free access is an act of solidarity, not a parallel healthcare system. Its purpose is to ensure that no patient is left without hope - while respecting the essential role and responsibility of public health systems. This is why this Policy exists: to define, with clarity, honesty and human warmth, how Fondazione Telethon behaves when a patient needs access to a therapy whether we distribute them directly or they are made available through a licensing partner – and for whatever reason cannot obtain it through ordinary channels.

2. Scope of This Policy: Our Commitment to Patients

This Policy applies to all therapies developed by Fondazione Telethon and commercialized directly or through licensing agreements with industrial partners, both before regulatory approval (pre-approval access) and after it (post-approval access).

It is dedicated to patients with rare genetic diseases for whom Telethon therapy may represent the only or best therapeutic option available, while also providing information to treating physicians, specialist centers, and patient organizations and advocacy groups so they can help guide and support patients.

This Policy does not replace or derogate from applicable local laws and regulations in any country, which always remain the primary reference. Nor does it constitute an automatic guarantee of access for any individual patient: every case is evaluated individually, with care and attention.

3. Pro-bono Access: Only When There is No Other Way

When a patient cannot access one of our therapies, Fondazione Telethon's first response is *"we will do everything possible to help you get the therapy through the right channels."*

This means that, together with patients and families, we commit to **exhausting every possible option for access**. We try to ensure that the institutions and organizations responsible for providing care - health systems, insurers, or regulatory bodies - have every opportunity to step in and do what they are meant to do.

Only when all reasonable options have truly been exhausted, and when the patient's situation qualifies for eligibility, does Fondazione Telethon consider activating a pro-bono access pathway.

#	Step	What we do
1	**Advocacy and negotiation**	*We activate all available channels to persuade governments, regulators, payers (and the licensee company if it is the case) to take responsibility for access.*
2	**Patient support**	*We work with patient organizations to help each individual navigate the available pathways in their country.*
3	**Pro-bono access**	*Only when the previous steps have not produced results, and the Foundation's financial sustainability allows it, do we supply the therapy free of charge.*

This approach applies both before and after a therapy is approved. Approval does not always mean access, and when barriers remain, we do not look away. Pro-bono access is always a last step, never a substitute for the responsibility of healthcare systems. Above all, our promise is simple: *we will stand with patients, try every possible path first, and act with care, honesty, and humanity at every step*.

4. Who Is Eligible for the Pro-Bono Programme

Pro-bono access is reserved for patients who simultaneously meet all the criteria listed below. These criteria exist not to exclude, but to ensure fairness, transparency and the long-term sustainability of the program, so that it can continue to exist over time.

4.1 Clinical Criteria

- The patient must have a serious, progressive or potentially life-threatening condition for which the Telethon therapy represents the only or best available option.
- All approved therapeutic alternatives must have been explored and reasonably ruled out.
- In the case of post-approval access, the condition must fall within the authorised indications of the therapy.
- The patient must have a referring physician and an active treatment plan, ensuring clinical supervision throughout treatment.
- The patient (or their legal guardian) must be capable of providing informed consent after being adequately informed of the risks, benefits and uncertainties associated with the therapy.

4.2 Payer Access Criteria

- The patient must not have access to the therapy through national health system coverage, private insurance or government subsidy programmes.
- All available channels (insurance reimbursements, assistance programmes, humanitarian funds, etc..) must have been pursued without success.

- Fondazione Telethon facilitates connections between local and international patient advocacy organisations, to support awareness and exploration of potential access pathway.

4.3 Geographic and Socioeconomic Criteria

- Eligible patients include those residing in countries where the therapy is not approved, or where it is approved but economically inaccessible due to structural barriers in the health system.
- Particular attention is given to patients in low-income countries and underprivileged populations, in line with our commitment to reducing global disparities in access to rare disease treatments.

5. How We Decide: Governance and Transparency

Pro-bono access decisions are never the result of external pressure, media visibility or geographic proximity. They follow a structured, fair and documented process.

5.1 Prioritization

Every pro-bono access request is evaluated and a **pro-bono waiting list** is defined based on:

- Medical need: Severity, life-threatening conditions, lack of alternatives
- Geographic and socio-economic factors

The pro-bono waiting list is reviewed every six months, to reassess cases based on changes in patients' clinical conditions, socio-economic circumstances, eligibility status, ensuring that the list remains accurate and up to date.

5.2 Independent Access Committee

Whenever the prioritization of cases based on the above-mentioned criteria poses any uncertainty, an **Independent Access Committee** might be consulted. Such a committee is composed of:

- specialist clinicians in the relevant therapeutic areas;
- bioethics and/or access experts;
- representatives of patient organizations and families.

The Committee re-evaluates the requests applying the criteria defined in this Policy, in complete independence from commercial or reputational considerations. All recommendations are documented and reasoned. The pro-bono waiting list can be consequently revised, based on the recommendations by the Independent Access Committee.

5.3 Final Decision and Financial Approval

The final decision to activate a pro-bono case rests with Fondazione Telethon, which verifies the availability of medicine, the patient's family's ability to cover hospitalization costs and the availability of dedicated funds. Every approved case requires the co-signature of the CEO, CFO and CSO (Chief Scientific Officer), ensuring rigorous oversight of the Foundation's resources.

6. Financial Sustainability: An Effort to Protect Access

Dedicated financial resources are allocated to ensure fair and sustainable access to therapies for patients. These resources are drawn from a specific fund established **exclusively for this purpose**. The fund is supported by positive financial results generated through the commercial distribution of therapies, and by net revenues from royalties deriving from therapies that are already developed and

marketed, as well as from those that may be developed and commercialized in the future. Ad hoc fundraising initiatives might also be launched to increase the size of the fund. Such initiatives must be transparent and explicit in their purpose while Fondazione Telethon general fundraising activities should not be directed to this fund.

> *If Fondazione Telethon exhausts its resources responding to today's pro-bono requests, it will not be able to fund the research that will produce tomorrow's therapies. Every patient we help today matters. Every patient we could help tomorrow matters just as much.*

7. Communication and Non-Promotional Posture

Fondazione Telethon is committed to managing the pro-bono programme with absolute communicative integrity.

This means:

- Ad hoc fundraising campaigns might be launched only in agreement with the patients and their families;
- pro-bono cases are never used for general promotional or fundraising purposes without the explicit consent of patients and families;
- the free supply of a therapy does not imply any assessment of its efficacy, nor does it constitute a commitment to reimbursement by health systems;
- Fondazione Telethon publishes an annual anonymised report on the number of patients served, their geographic distribution and the overall impact of the programme;
- a strictly non-promotional posture is maintained: clinical data collected serves science, not commercial communication.

8. Review and Continuous Improvement

This Policy is a **living document**. It will be **reviewed at least every 12 months** (validity period) by an external, independent Advisory Board, which will assess the fairness of the process, the equity of access criteria and the program's impact on patients.

The Board may issue binding recommendations for revising criteria and procedures. Fondazione Telethon commits to publishing its annual conclusions in a clear and accessible format, including for patients and families.

During its validity period, this Policy may be adapted in response to regulatory, scientific or operational changes. Any substantial modification will be communicated promptly to all relevant stakeholders.

A commitment rooted in our history

Fondazione Telethon was born from the strength of families. It continues to exist thanks to the generosity of millions of people who every year choose to support research into rare genetic diseases. This Policy is our way of honouring that trust: being rigorous in our processes, fair in how we allocate resources, and faithful to our original commitment to leave no one behind.