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AIFA Board approves the call for independent research on rare diseases for 2025

At its extraordinary meeting on 10 April, the Board of Directors of the Italian Medicines Agency approved the call for independent research on rare diseases for the year 2025. The call is addressed to all Italian researchers in organisations and institutions wishing to conduct non-commercial studies and it is financed by a portion of the Fund made up of the contribution from promotional expenses incurred annually by pharmaceutical companies.

The call provides for the allocation of €17,800,000 and it represents a further step in AIFA's commitment to promoting independent scientific research, with a particular focus on low-incidence diseases, which often do not attract significant commercial investment. The main objective is to stimulate the development of effective drug therapies for rare diseases, improving the health and quality of life of patients. The 2% Fund is established by the Consolidated Text on Rare Diseases (Law 175/2021).

AIFA President Robert Nisticò stressed that 'promoting and supporting independent research has always been part of the mission of AIFA, the first Medicines Agency in Europe to include such scope among its institutional objectives, particularly in strategic sectors and in areas of potentially low interest for profit research, such as rare diseases. Hope, dignity and innovative treatments are rights to be guaranteed to every patient with these diseases. The approval of the call for proposals goes in this direction, with the intention of incentivising projects and studies that generate evidence with concrete benefits for patients: a commitment that the Agency pursues with responsibility and determination'.

Marcello Gemmato, Health Undersecretary with responsibility for rare diseases, said that 'Italy is confirmed as one of the best countries in the world for its approach to rare diseases and the approval of AIFA's call for independent research in such specific field is proof of this. The call is in line with the provisions of the Consolidated Text on Rare Diseases, which has earmarked specific resources for non-profit research on orphan drugs. Above all, the call focuses on the health needs of people with rare diseases by incentivising projects and clinical studies aimed at identifying possible new treatments.' The undersecretary concluded by claiming that ' I am certain that AIFA will guarantee timeliness and rigour in the publication of the call for proposals and in the evaluation of proposals, in the primary interest of patients and their families.'

In particular, as provided for in the Consolidated Text on Rare Diseases, the AIFA call concerns two research lines: on the one hand preclinical and clinical studies aimed at the development of pharmacological therapies for diseases lacking specific treatments, including projects for repositioning existing drugs aimed to investigate and support new therapeutic indications in rare diseases. On the other hand, the call concerns preclinical and clinical studies aimed at the development of orphan medicines derived from plasma.

The 2025 call will be available on AIFA's institutional portal in June and it will include details on eligibility criteria and how to submit applications.