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AIFA supports the Rare Disease Day 2026

The rarity of a disease cannot result into invisibility. For those living with a rare disease, the first obstacle is not only the treatment itself, but the journey: obtaining a diagnosis, finding a disease reference centre, accessing appropriate care. The ability of institutions to guarantee equity, continuity of care, and access to innovative therapies is measured just in this complexity.

“Rare diseases, taken together, affect over 2 million citizens in Italy and around 30 million in Europe. These are often chronic and debilitating conditions, for which the average time to reach a diagnosis can exceed four years. These figures push us to strengthen treatment pathways, ensuring fair and timely access to innovative therapies,” says AIFA President Robert Nisticò.

With this in mind, AIFA headquarters will be illuminated with the colours symbolizing the initiative on February 28 - Rare Disease Day, in order to shine a spotlight on a reality that cannot remain invisible.

On an operational level, the Agency continues to work on the assessment of and access to innovative therapies, a key element in strengthening the response to patients' needs in a tangible way. This is the context for the new therapeutic opportunities authorized at European level. “In Italy, just in the last few days, we have established reimbursement for two innovative drugs: Duvyzat for Duchenne muscular dystrophy, and Carvykti, the first CAR-T authorised for multiple myeloma,” continues President Nisticò. In 2025, the European Medicines Agency also authorized an ex vivo gene therapy to correct the genetic defect underlying Wiskott-Aldrich syndrome, a rare immune disorder for which treatment options have been extremely limited until now. An innovative topical gene therapy is also on the way for the treatment of wounds in patients with dystrophic epidermolysis bullosa, a severe and debilitating genetic skin disease.
A further important therapeutic option for Graves' eye disease, a rare autoimmune condition that can significantly compromise quality of life, is a monoclonal antibody.

Lighting AIFA’s building on February 28 is not only a symbolic gesture, but a sign of a commitment that concerns the organization of care pathways, drug governance, sustainability, and the concrete protection of the right to health of people with rare diseases.

“It is our duty to ensure that every person with a rare disease receives appropriate care and to work to ensure that no patient feels alone,” concludes President Nisticò.