aaplizumab for Hodgkin lymphoma/non-Hodgkin lymphoma, ruxolitinib for myeloproliferative neoplasm BCR/JAK2-rearranged, blinatumomab for acute lymphoblastic leukaemia, ruxolitinib for graft versus host disease); 3 requests (12%) came from the oncological/gynaecological area (trabectedina for tube ovarian carcinoma and serous ovarian adenocarcinoma) and 1 (5%) from the ophthalmology area (cenegermin for neurotrophic keratitis). Eight of 24 authorised patients (33%) are still receiving treatment and 16 (67%) have completed their treatment programme. Of note, 16/23 (70%) oncologic patients had a disease response; moreover, 4/9 (44%) high risk acute leukaemia patients have undergone bone marrow transplant. The total cost of the authorised treatments was about € 700 000, of which € 142 000 was already credited back to the hospital.

Conclusion and relevance These results demonstrate that Fondo 5% represents a scientific based method guaranteeing access to highly expensive therapeutic programmes, impacting on patient survival, without affecting the cost effectiveness balance and sustainability of the national health system.