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ENVISIONING SUSTAINABILITY IN PERSONALISED MEDICINE: FONDO AIFA 5% AND THE ITALIAN EXAMPLE

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Background and Importance. Sustainability in the era of personalized medicine represents one the major problems due to a possible limited access to innovative therapies. Since 2003 the Agenzia Italiana del Farmaco (AIFA), along with the contribution of pharma industries, has established an innovative and unique program, namely “Fondo 5%” (1). The aim of this project is to allow innovative and highly expensive treatments to rare-disease patients. All these therapies have been approved by the European medicine agency (EMA) but before the AIFA authorization and reimbursement for the specific indication. A joined evaluation by physicians and clinical pharmacists, based on scientific literature, clinical reports, treatment plan and cost-estimate analysis, generates a patient-specific request for a peculiar drug not otherwise available through the conventional ways. AIFA is responsible for the scientific evaluation, the final authorization or rejection. Once the treatment plan has been approved by AIFA, the clinician is authorized to administer the therapy whose cost will be subsequently refunded by AIFA (2).

Aim and Objectives. To describe the Italian experience in order to improve the availability of the best, innovative therapies considering sustainability of the NHS.

Materials and Methods. Collecting and process of drug request for Fondo 5% and analysis of the clinical and economic impact.

Results. Period: Aug 2018-Sept 2019. 24 treatments authorized by AIFA. 70% oncologic patients with a disease response; moreover, 44% of AML have been undergone to bone marrow transplant. Total amount of authorized treatments: € 700.000, of which € 142.000 already credited back to the hospital.

PATIENT	CLINICAL AREA	DRUGS	DIAGNOSIS	STATE OF TREATMENT	OUTCOME
1	GYNECOLOGICAL	Trabectedine	Ovaric carcinoma	ON GOING	EARLY RESPONCE
2	HEMATOLOGICAL	Venetoclax	AML	ON GOING	EARLY RESPONCE
3	HEMATOLOGICAL	Eltrombopag	Pure Aplasia	ON GOING	EARLY RESPONCE
4	HEMATOLOGICAL	Ruxolitinib	GVHD	ON GOING	EARLY RESPONCE
5	HEMATOLOGICAL	Ruxolitinib	Myeloproliferative neoplasm BCR-JAK2	ON GOING	EARLY RESPONCE
6	HEMATOLOGICAL	Ruxolitinib	GVHD	ON GOING	EARLY RESPONCE
7	HEMATOLOGICAL	Pembrolizumab	Hodgkin Lymphoma	ON GOING	EARLY RESPONCE
8	HEMATOLOGICAL	Venetoclax	MCL	ON GOING	EARLY RESPONCE
9	HEMATOLOGICAL	Pembrolizumab	Hodgkin Lymphoma	EOT	COMPLETE RESPONCE
10	HEMATOLOGICAL	Blinatumumab	ALL	EOT	COMPLETE RESPONCE
11	HEMATOLOGICAL	Venetoclax	AML	EOT	PROGRESSION DISEASE

PATIENT	CLINICAL AREA	DRUGS	DIAGNOSIS	STATE OF TREATMENT	OUTCOME
12	HEMATOLOGICAL	Venetoclax	AML	EOT	TOXICITY
13	GYNECOLOGICAL	Trabectedine	Ovaric carcinoma	EOT	COMPLETE RESPONCE
14	HEMATOLOGICAL	Venetoclax	AML	EOT	PROGRESSION DISEASE
15	HEMATOLOGICAL	Venetoclax	AML	EOT	COMPLETE RESPONCE
16	HEMATOLOGICAL	Venetoclax	AML	EOT	PROGRESSION DISEASE
17	HEMATOLOGICAL	Venetoclax	AML	EOT	COMPLETE RESPONCE
18	HEMATOLOGICAL	Venetoclax	AML	EOT	COMPLETE RESPONCE
19	HEMATOLOGICAL	Blinatumumab	ALL	EOT	COMPLETE RESPONCE
20	HEMATOLOGICAL	Venetoclax	AML	EOT	TOXICITY
21	HEMATOLOGICAL	Pembrolizumab	PMBCL	EOT	PROGRESSION DISEASE
22	GYNECOLOGICAL	Trabectedine	Ovaric adenocarcinoma	EOT	PROGRESSION DISEASE
23	HEMATOLOGICAL	Blinatumumab	ALL	EOT	COMPLETE RESPONCE
24	OPHTHALMOLOGY	Cenegermin	Neurotrophic keratitis	EOT	COMPLETE RESPONCE

Conclusion and Relevance. These results demonstrate that Fondo 5% represents a scientific-based method able to guarantee the access to highly expensive therapeutic programs, impacting patients survival (96%), without affecting the cost-effectiveness balance and the NHS sustainability.

References and/or Acknowledgements

(1) L.326/2003. (2) www.aifa.gov.it/fondo-nazionale-aifa

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No conflict of interest. Contact: f.bocchio@smatteo.pv.it