

EXPENSIVE AND POORLY SUSTAINABLE DRUGS: STRATEGIES TO MANAGE THE COSTS

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INTRODUCTION

Over the past decades, many new and highly expensive drugs for rare diseases (RD) entered the market. (Table 1) Their high cost is generally related to the research and technology behind their development.

Italian Healthcare System (*Servizio Sanitario Nazionale - SSN*) has set basic levels of care (LEA) which define as necessary a pool of medicines for the treatment of various pathologies. The costs of these drugs are entirely met by SSN, and this represents a huge expense which requires to put in place effective strategies to ensure the system to work: the Italian Medicine Agency (AIFA) plays a key role by cooperating with pharmaceutical industries to manage the costs of medicines, ensuring their access to patients. At regional level, each Italian region is responsible for the administration of its own Healthcare system (*Servizio Sanitario Regionale - SSR*); funded by the government to cover the basic levels of care, Regions have to identify strategies to manage the pharmaceutical expenditure.

Drug	Disease	Pts RER	Year 1st commercialisation
Edaravone	ALS	30*	2017
Chenodeoxycholic Acid	Cerebrotendinous xanthomatosis	3§	2017
Ataluren	DMD	63*	2017
Agalsidase beta	Fabry's disease	83§	2003
Agalsidase alfa			2006
Migalastat			2017
Imiglucerase	Gaucher's disease	40§	2008
Turoctocog alfa	Haemophilia A	356§	2014
Simoctocog alfa			2015
Efmoroctocog alfa			2016
Nonacog gamma	Haemophilia B	99§	2017
Eftrenonacog alfa			2017
Albutrepenonacog alfa			2017
Eculizumab	Hemolytic-uremic syndrome	28§	2008
Icatibant	Hereditary angioedema	59§	2009
Human C1 esterase inhibitor			2016
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Pirfenidone	Idiopathic pulmonary fibrosis	80*	2013
Nintedanib			2016
Amifampridine	Lambert-Eaton myasthenic syndrome	11§	2012
Idebenone	Leber's hereditary optic neuropathy	92*	2016
Idursulfase	Mucopolysaccharidosis II	5§	2011
Eculizumab	Paroxysmal nocturnal hemoglobinuria	10§	2008
Alglucosidase alfa	Pompe's disease	22§	2007
Nusinersen	SMA	20*	2016

* Estimated as eligible

§ Regional Rare diseases register data

Table 1. New drugs for RD

METHODS

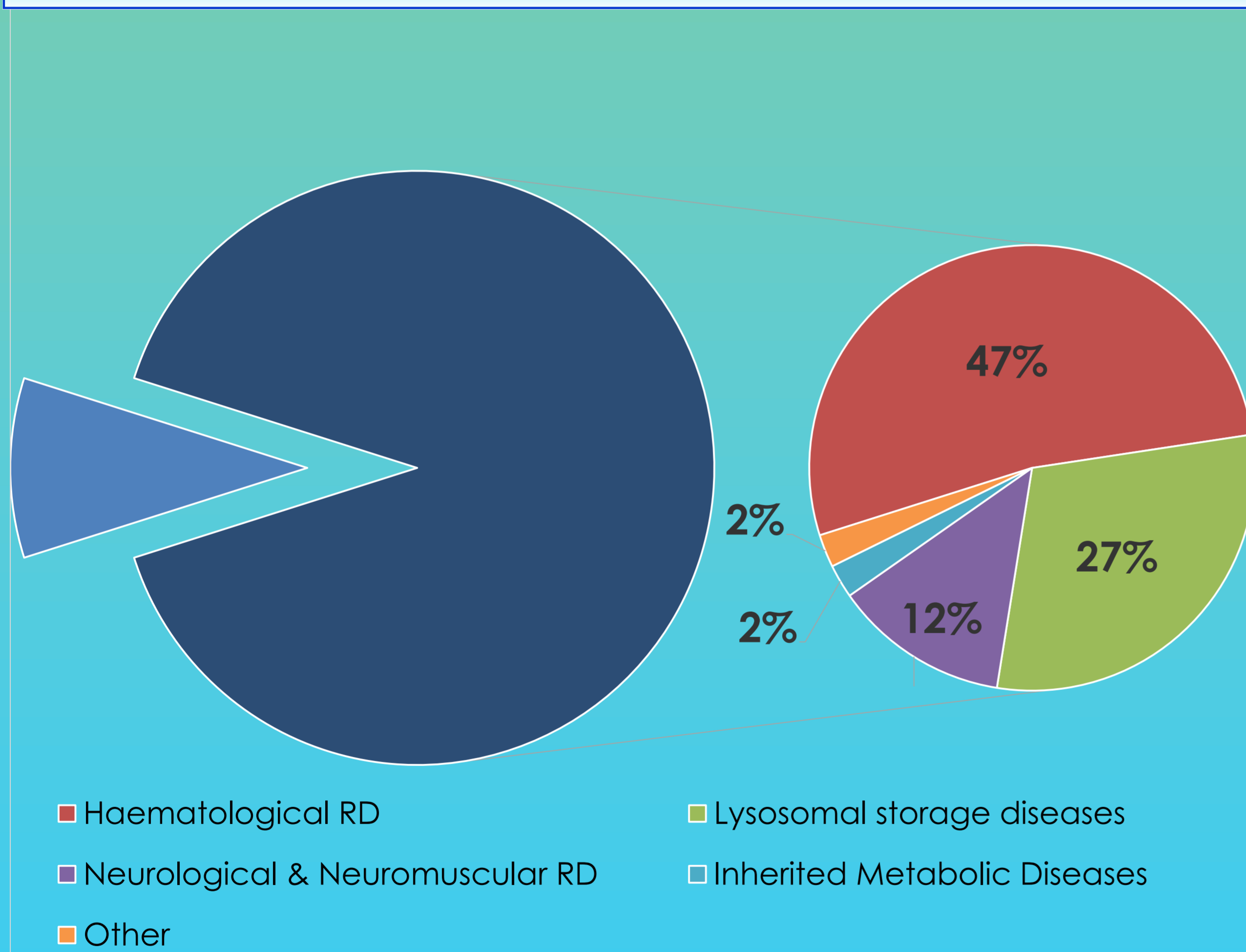


Figure 1. Costs in RER for RD drugs per RD groups

In 2017, Emilia-Romagna region (RER) spent **43 mln € for RD drugs**, the 90.2% of which was due to twenty-five bioactive molecules for the treatment of four groups of RD. (Figure 1) On the basis of these evidences, RER has been developing strategies to effectively keep under control the costs associated to these therapies.

1. Pharmacological protocols

Many drugs and supplements included in RD therapies exceed LEA, thus patients are charged for their costs. Yet, in order to guarantee free access only to effective drugs, groups of experts have been created to peer-review the pharmacological therapies and determine the medicines whose therapeutic efficacy and safety are scientifically proven. These drugs are then covered by the Region.

2. Careful control of the medicines included in LEA

RER put together groups of expert clinicians which act as supervisors of the therapies for the treatment of RD. Their role is to double-check inclusion and exclusion criteria for drugs to ensure therapy efficacy and safety, as well as address the correct segment of patient to treat.

CONCLUSIONS

Nowadays new therapies for rare diseases are (fortunately) becoming available, but the associated costs often represent a burden for SSR. Thus, it becomes critical to identify effective strategies to guarantee the economic sustainability of SSR and SSN by careful monitoring of the expenses for orphan drugs as well as ensure the appropriate prescription of these medicines.

One challenge for SSR and SSN is to identify - the diagnosis being equal - the criteria to grant the required therapy to those patients who can really benefit from it. In this regard, it is essential to share these goals with all stakeholders (Public Health Services, clinicians, patients' associations) to ensure appropriate care for all patients who need it.



For further information please visit the site
<http://salute.regione.emilia-romagna.it/assistenza-ospedaliera/malattie-rare>
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