

# 2021 Access Report

## on Orphan Drugs in Spain

Analysis of all trade-named ODs with European orphan designation in force as of December 31, 2021.

deLmhu



01

Out of over 2,000 active substances with orphan designation by the Committee for Orphan Medicinal Products and Orphan Products (COMP) of the European Drugs Agency (EMA), we have identified those active substances with a valid orphan designation as of December 31, 2021 (approx. 1,730). OD designation is granted during the first stages of a drug's research, so that within the active substances that have a valid OD designation, those with a commercial name (more than 200) are identified in each report and, of these, duplicates are eliminated because they have several indications. By eliminating these duplicates, **176 ODs**<sup>1</sup> **with orphan designation** in force as of December 31, 2021 and with a trade name are obtained.

02

Afterwards, we analyzed which of these ODs have been withdrawn (or have not yet been included) in the European Commission's Community Register of Orphan Medicinal Products for marketing in the European Union (n=47). As a result, we obtained the **number of ODs with marketing authorization (MA)** as of December 31, 2021, in European countries for their commercialization (**n=129**).<sup>2</sup>

03

The third step has consisted in identifying which of those ODs with a commercial name that are approved for marketing in the European Union have **National Code (NC)** authorization by the AEMPS for marketing in Spain (**n=111**).<sup>3</sup>

04

Lastly, we identified which ODs were **NHS funded** and the reimbursement date, according to the information contained in the Nomenclator of the Ministry of Health (**n=56**). The first date of discharge has been taken as the funding date.<sup>4</sup>

Note: Throughout the document, the laboratory of each OD is the titular laboratory available in the [Ministry of Health Nomenclator](#). If the laboratory offering the NHS is different, the two laboratories are indicated. In the ODs not available in the Nomenclator, it is the laboratory that appears in the EMA.

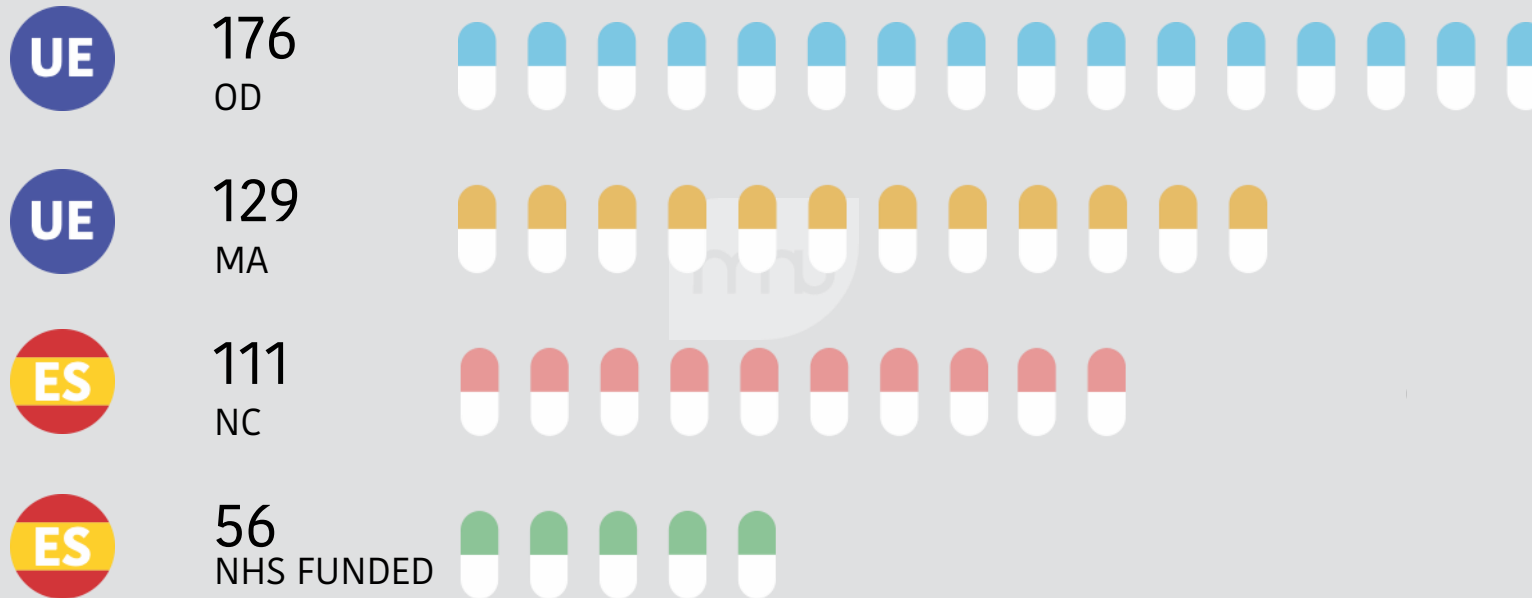


# Orphan Drugs

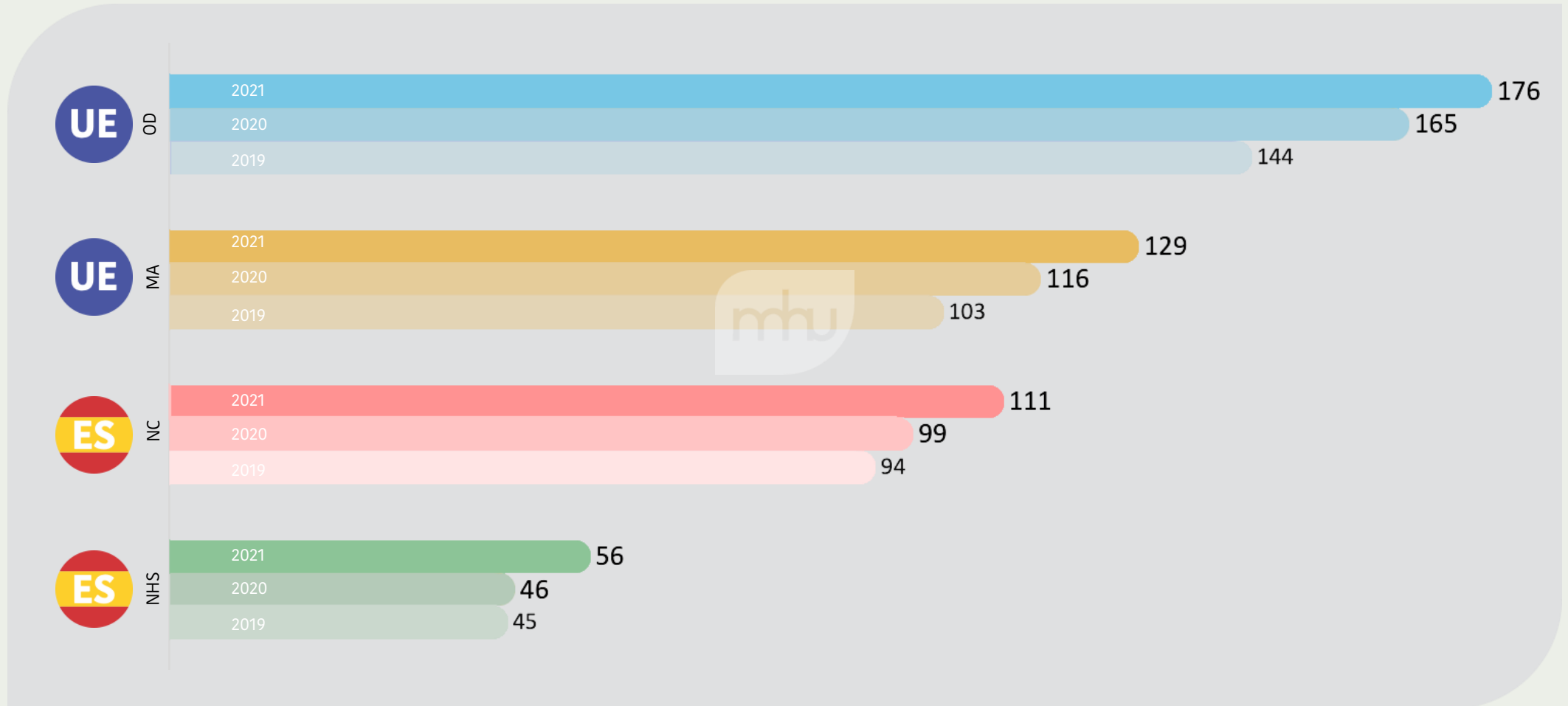
European Union  
and Spain

2021

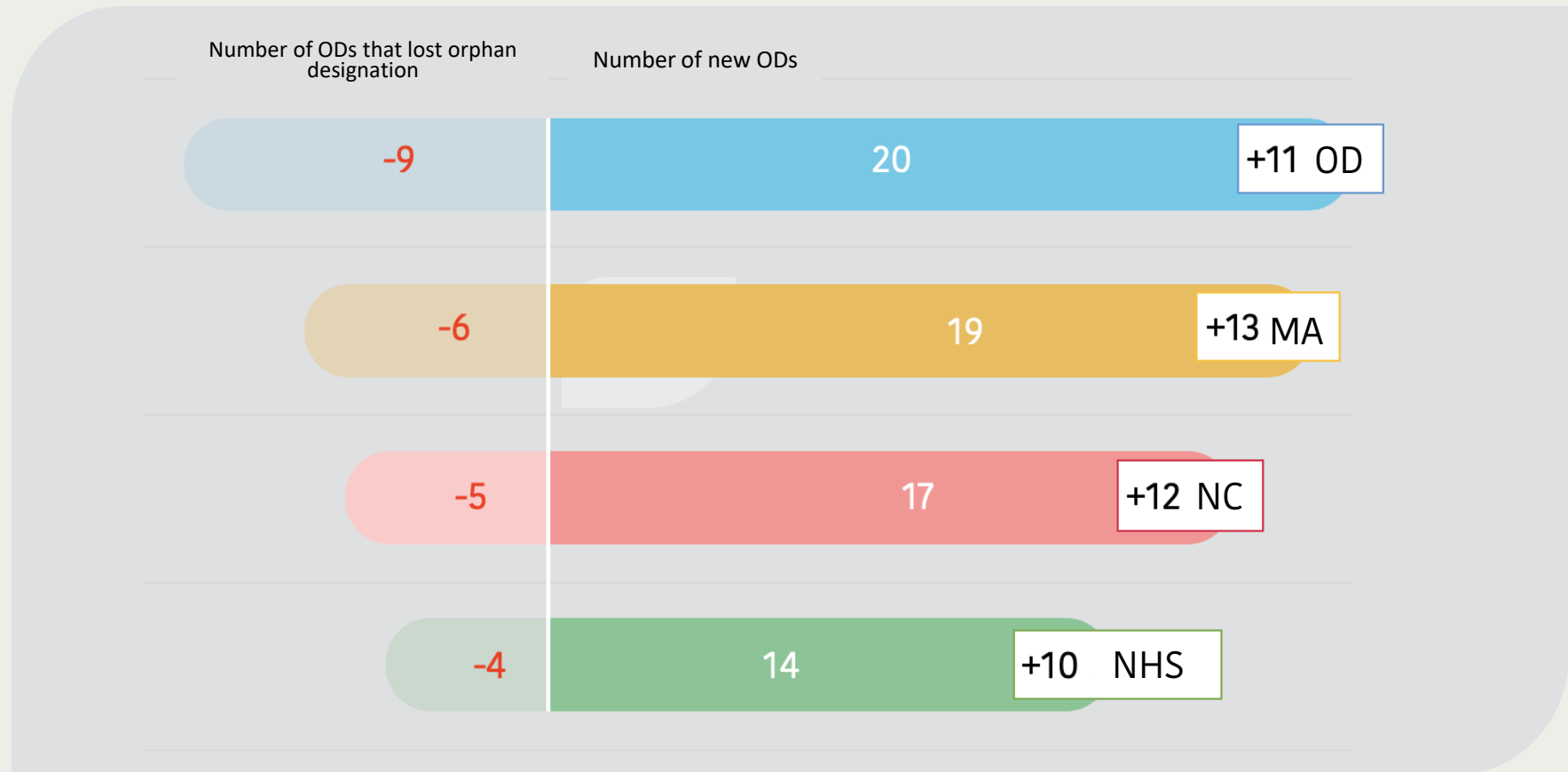
# Main indicators EU and Spain



# Evolution of indicators 2019-2021



# Variation of indicators during 2021





# Details of the variation in orphan designations

	2021 vs 2020	New ODs	Laboratoy	Comments
	+ 20 ODs have obtained orphan designation	ENSPRYNG EVRYSDI KOSELUGO PEMAZYRE SOGROYA ABECMA BRUKINSA BYLVAY FLYNPOVI IMCIVREE MINJUVI NEXVIADYME SKYSONA VOXZOGO ASPAVELI LONAPEGOMATROPIN	Roche Roche AstraZeneca Incyte Novo Nordisk Celgene BeiGene Albireo Cancer Prevention Pharma Rhythm Pharmaceuticals Incyte Genzyme Europe BV BlueBird Bio BioMarin International SOBI Ascendis Pfizer Global Blood Therapeutics Deciphera Viela	CHMP + 23/04/2021 CHMP+ 26/02/2021 CHMP+ 23/04/2021 CHMP+ 25/02/2021 CHMP+ 1/02/2021 CHMP 25/06/2021 No CHMP CHMP 21/05/2021 CHMP negative CHMP 21/05/2021 CHMP 25/06/2021 CHMP 23/07/2021 CHMP 21/05/2021 CHMP 24/06/2021 CHMP 15/10/2021 CHMP 11/11/2021 CHMP 16/12/2021 CHMP 16/12/2021 CHMP 17/09/2021 CHMP 11/11/2021
	- 9 ODs have lost orphan designation	ESBRIET SIBNAYAL CARBAGLU GLYBERA LUMOXITI MOZOBIL IMBRUVICA PLENADREN BRUKINSA	Roche Advicenne Recordati Rare Diseases UniQure biopharma B.V. AstraZeneca Genzyme Europe BV Janssen Shire BeiGene	Loss OD designation Loss OD designation Loss OD designation Withdrawn Withdrawn Loss OD designation Loss OD designation Loss OD designation Loss OD designation

# Detail of the variation in marketing authorizations

	2021 vs 2020	New ODs	Laboratoy	Comments
	<p><b>+ 19 ODs</b> have obtained MA</p>	<p>ADAKVEO EVRYSDI HEPCLUDEX INREBIC LUMOXITI PEMAZYRE ABECMA BYLVAY ELZONRIS ENSPRYNG IMCIVREE KOSELUGO SKYSONA SOGROYA ARTESUNATE ASPAVELI MINJUVI QINLOCK VOXZOGO</p>	<p>Novartis Roche Gilead BMS AstraZeneca Incyte Celgene Albireo AB Stemline Therapeutics Roche Rhythm Pharmaceuticals AstraZeneca AB BlueBird Bio Novo Nordisk Amivas SOBI Incyte Deciphera BioMarin</p>	<p>MA 28/10/2020 MA 26/03/2021 MA 31/07/2020 MA 08/02/2021 MA 08/02/2021 MA 26/03/2021 MA 18/08/2021 MA 16/07/2021 MA 07/01/2021 MA 24/06/2021 MA 16/07/2021 MA 19/06/2021 MA 16/07/2021 MA 31/03/2021 MA 22/11/2021 MA 13/12/2021 MA 26/08/2021 MA 18/11/2021 MA 26/08/2021</p>
	<p><b>- 6 ODs</b> have lost orphan designation</p>	<p>ESBRIET CARBAGLU LUMOXITI MOZOBIL IMBRUVICA PLENADREN</p>	<p>Roche Recordati Rare Diseases AstraZeneca Genzyme Europe BV Janssen Shire</p>	<p>Loss OD designation Loss OD designation Withdrawn Loss OD designation Loss OD designation Loss OD designation</p>



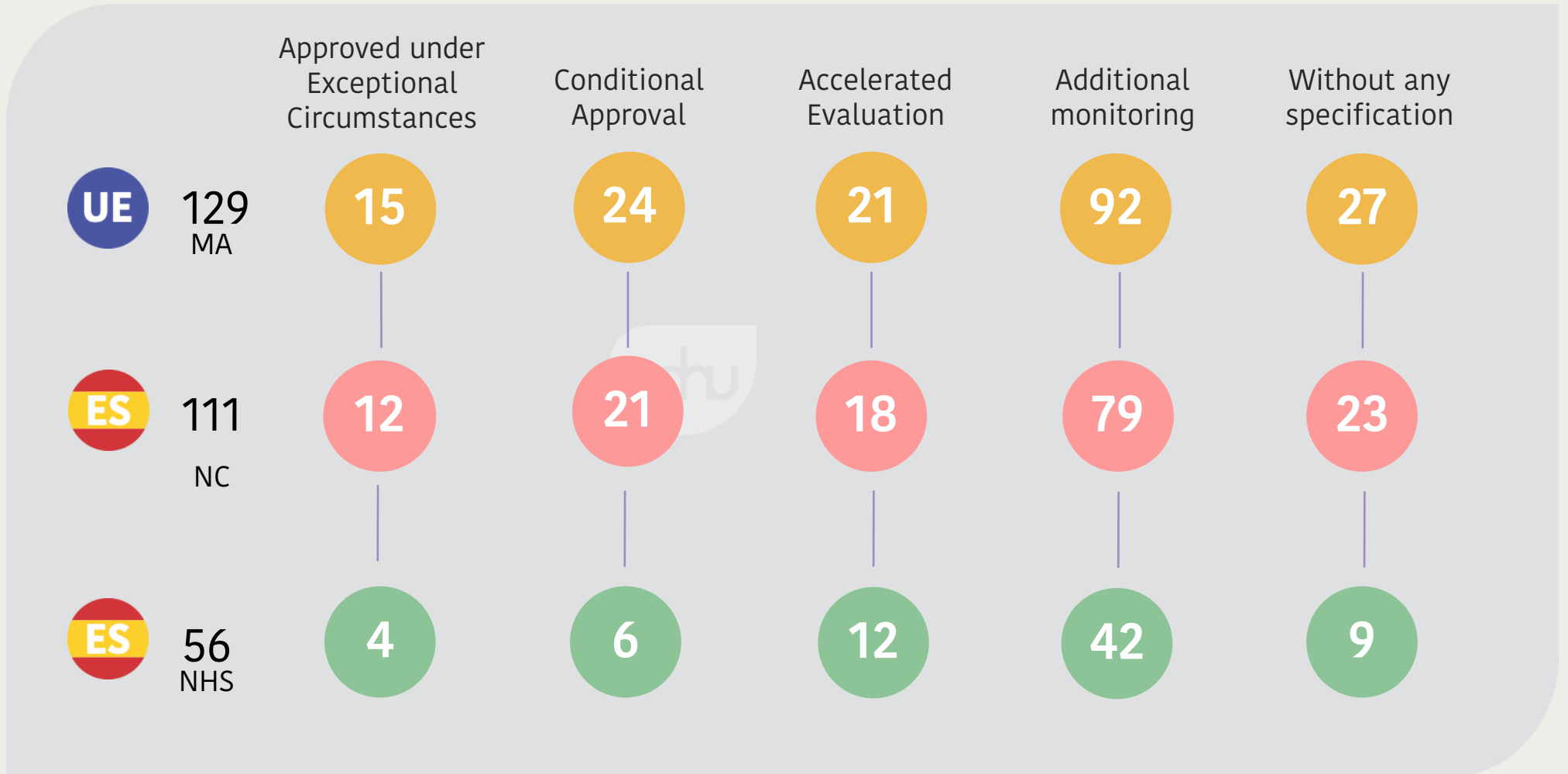
## Detail variation of national codes

	2021 vs 2020	New ODs	Laboratoy	Comments
<p>NC</p>	<p>+ 17 ODs have obtained NC</p>	<p>ADAKVEO AMGLIDIA EVRYSDI INREBIC ISTURISA TRECNDI TREPULMIX ENSPRYNG FINTEPLA KOSELUGO PEMAZYRE ASPAVELI BYLVAY LIBMELDY MINJUVI HEPCLUDEX IDEFIRIX</p>	<p>Novartis Ammtek Roche BMS Recordati Rare Diseases MBH Scipharm Roche Zogenix Roi (MSD) AstraZeneca Incyte SOBI Albireo Orchard Therapeutics Incyte Gilead Hansa</p>	<p>NC 01/02/2021 NC 14/01/2021 NC 21/04/2021 NC 26/02/2021 NC 12/02/2021 NC 03/02/2021 NC 07/04/2021 NC 26/07/2021 NC 02/06/2021 NC 27/07/2021 NC 07/05/2021 NC 30/12/2021 NC 21/12/2021 NC 19/10/2021 NC 18/09/2021 NC 16/10/2021 NC 09/12/2021</p>
	<p>- 5 ODs have lost orphan designation</p>	<p>ESBRIET CARBAGLU MOZOBIL IMBRUVICA PLENADREN</p>	<p>Roche Recordati Rare Diseases Genzyme Europe BV Janssen Shire</p>	<p>Loss OD designation Loss OD designation Loss OD designation Loss OD designation Loss OD designation (before its funding)</p>

# Detail of variation of funded orphan drugs

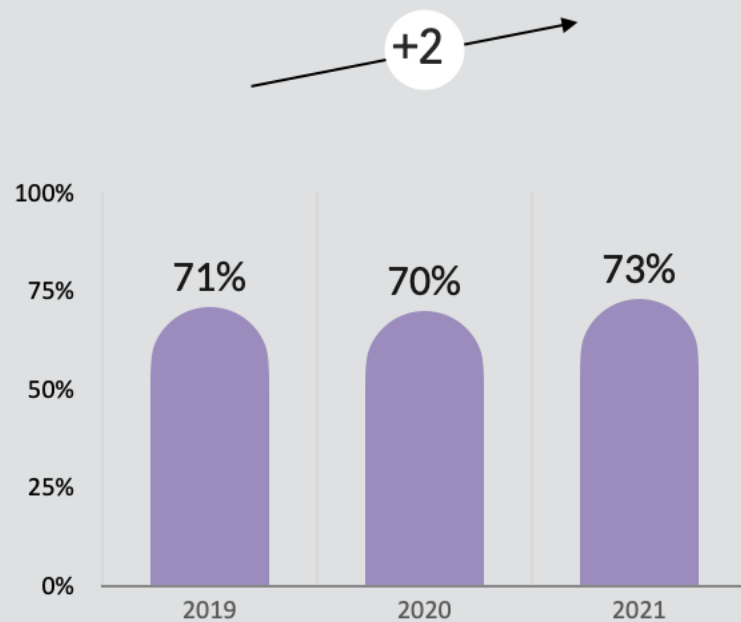
	2021 vs 2020	New ODs	Laboratoy	Comments
	<p>+ 14 ODs funded</p>	<p>CRYSVITA TAKHZYRO CABLIVI LUXTURNA MEPSEVII POTELIGEO PREVYMIS TREPULMIX QUENODESOXYCHOLIC ACID EPIDYOLEX GIVLAARI KAFTRIO POLIVY ZOLGENSMA</p>	<p>Kyowa Kirin Takeda Ablynx (Sanofi) Novartis Ultragenyx Kyowa Kirin MSD Scipharm/AOP Oprhan Leadiant GW Alnylam Vertex Roche Novartis</p>	<p>Entry date 01/01/2021 Entry date 01/03/2021 Entry date 01/07/2021 Entry date 01/05/2021 Entry date 01/07/2021 Entry date 01/07/2021 Entry date 01/08/2021 Entry date 01/08/2021 Entry date 01/12/2021 Entry date 01/09/2021 Entry date 01/09/2021 Entry date 01/12/2021 Entry date 01/09/2021 Entry date 01/12/2021</p>
	<p>- 4 ODs have lost orphan designation</p>	<p>ESBRIET CARBAGLU MOZOBIL IMBRUVICA</p>	<p>Roche Recordati Rare Diseases Genzyme Janssen</p>	<p>Loss OD designation Loss OD designation Loss OD designation Loss OD designation</p>

# ODs authorized under special conditions

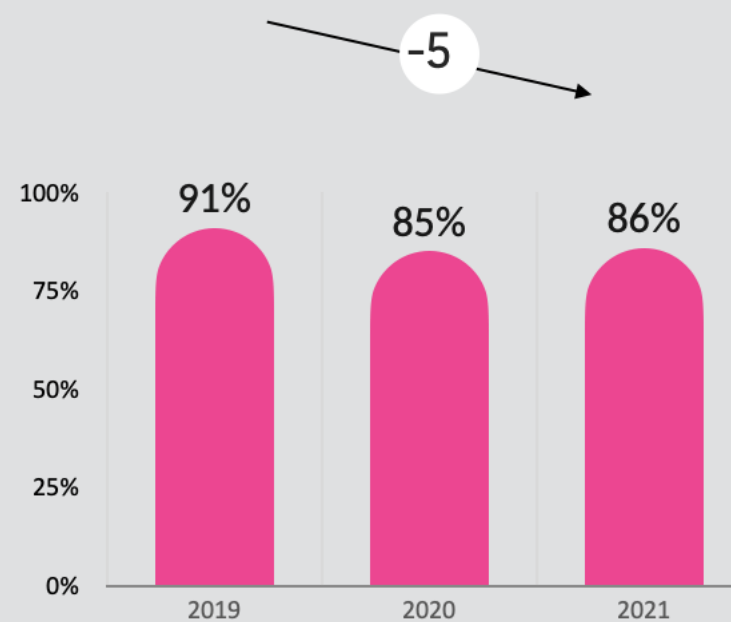


# Comparison between indicators 2019 - 2021

% of ODs with orphan designation and MA

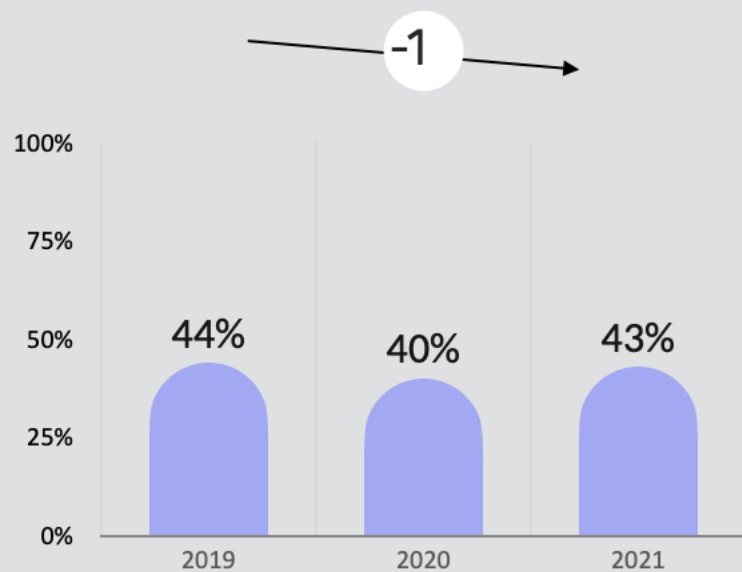


% of ODs with MA and NC

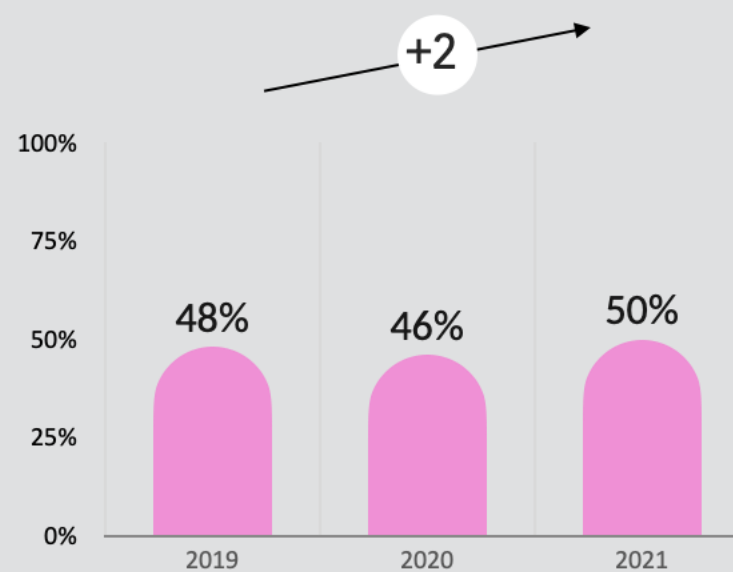


# Comparison between indicators 2019 - 2021

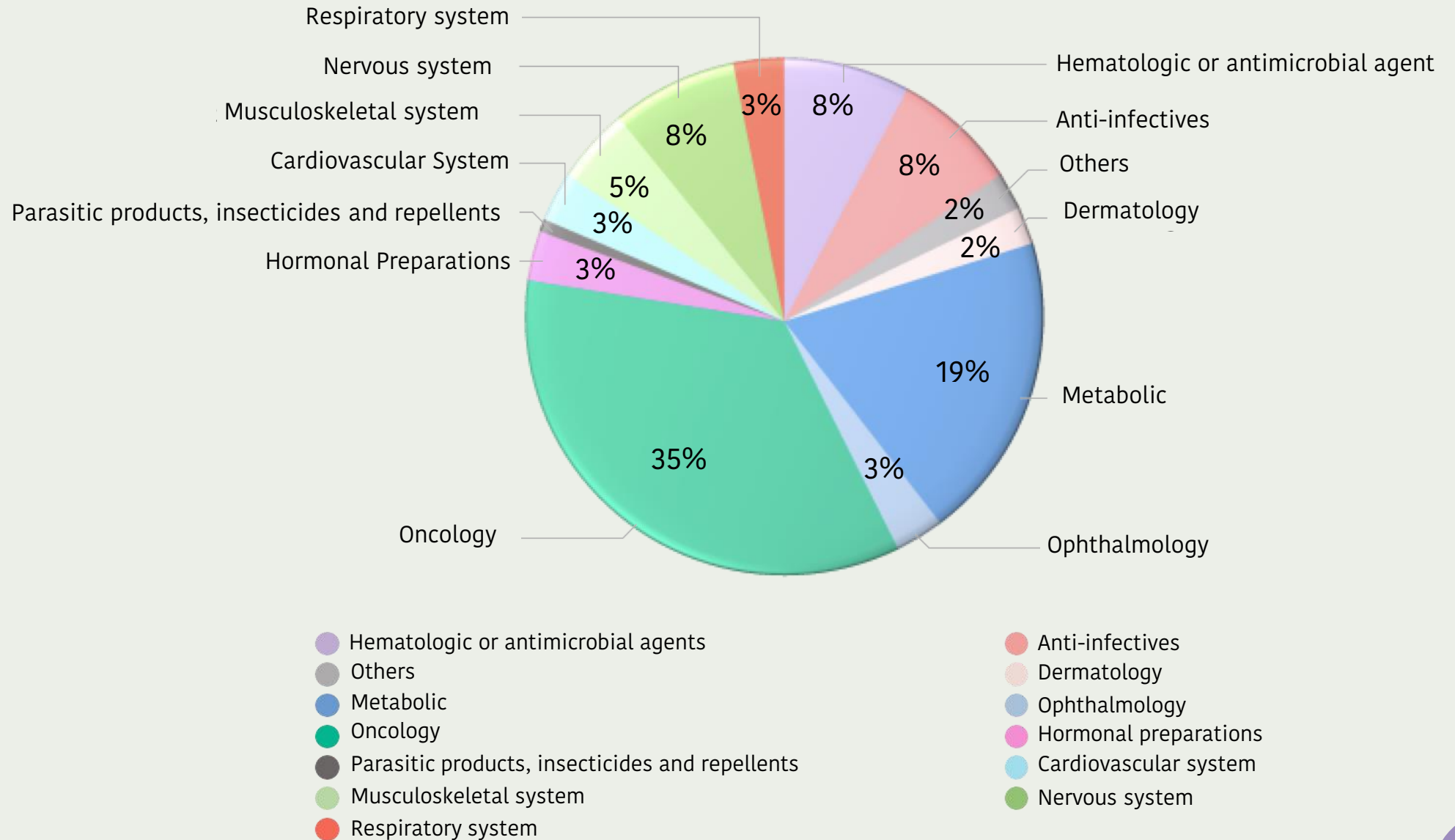
% of ODs with MA NHS funded



% of ODs with NC NHS funded



# Orphan drugs with MA by therapeutic area







## ODs with MA vrs NC



In 2021, el **85%** of the ODs that have MA have reached in Spain.  
**18** drugs still have no NC.



## Detailed information of the 18 ODs with MA without NC

Commercial Name	Date MA	Months of waiting (MA – 31/12/2021)	Therapeutic Area	Advanced Therapy
<b>1. ABECMA</b>	<b>MA 18/08/2021</b>	5	<b>Oncology</b>	
<b>2. ARIKAYCE</b>	MA 27/10/2020	14	Anti-infective	
<b>3. ARTESUNATE</b>	MA 22/11/2021	1	Parasitic products	
<b>4. AYVAKYT</b>	MA 24/09/2020	15	Oncology	
<b>5. COAGADEX</b>	MA 16/03/2016	71	Hematological agent	
<b>6. DEFITELIO</b>	MA 18/10/2013	100	Hematological agent	
<b>7. DOVPRELA</b>	MA 31/07/2020	17	Anti-infective	
<b>8. ELZONRIS</b>	MA 07/01/2021	12	Oncology	
<b>9. HETLIOZ</b>	MA 03/07/2015	79	Nervous system	
<b>10. IMCIVREE</b>	MA 16/07/2021	6	Metabolic	
<b>11. KETOCONAZOLE</b>	MA 18/11/2014	87	Dermatological	
<b>12. OBILTOXAXIMAB</b>	MA 18/11/2020	14	Anti-infective	
<b>13. QINLOCK</b>	MA 18/11/2021	1	Oncology	
<b>14. SKYSONA</b>	<b>MA 16/07/2021</b>	6	<b>Nervous system</b>	
<b>15. SOGROYA</b>	MA 16/07/2021	6	Hormonal preparations	
<b>16. STRIMVELIS</b>	<b>MA 26/05/2016</b>	68	<b>Others</b>	
<b>17. VOXZOGO</b>	MA 26/08/2021	4	Musculoskeletal system	
<b>18. ZYNTEGLO</b>	<b>MA 29/05/2019</b>	32	<b>Hematological agent</b>	



An aerial night view of a city street corner, likely in Madrid, Spain. The central focus is a large, ornate building with a prominent dome topped by a statue. The building's facade is highly detailed with classical architectural elements. A sign on the building reads "METROPOLIS". The street is illuminated by streetlights, and several cars are visible in motion. The surrounding cityscape is dense with buildings, and the overall atmosphere is that of a vibrant urban environment at night.

# Orphan drugs

SPAIN  
2021

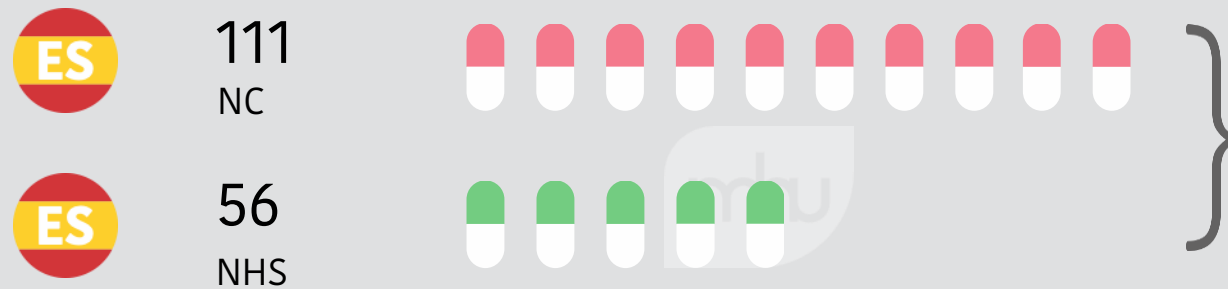


## Number of ODs with MA vrs NHS funded



In 2021, **43%** of ODs with MA are currently funded by the NHS, three points higher than in 2020 (40%).

## Comparison of ODs with NC and NHS funded

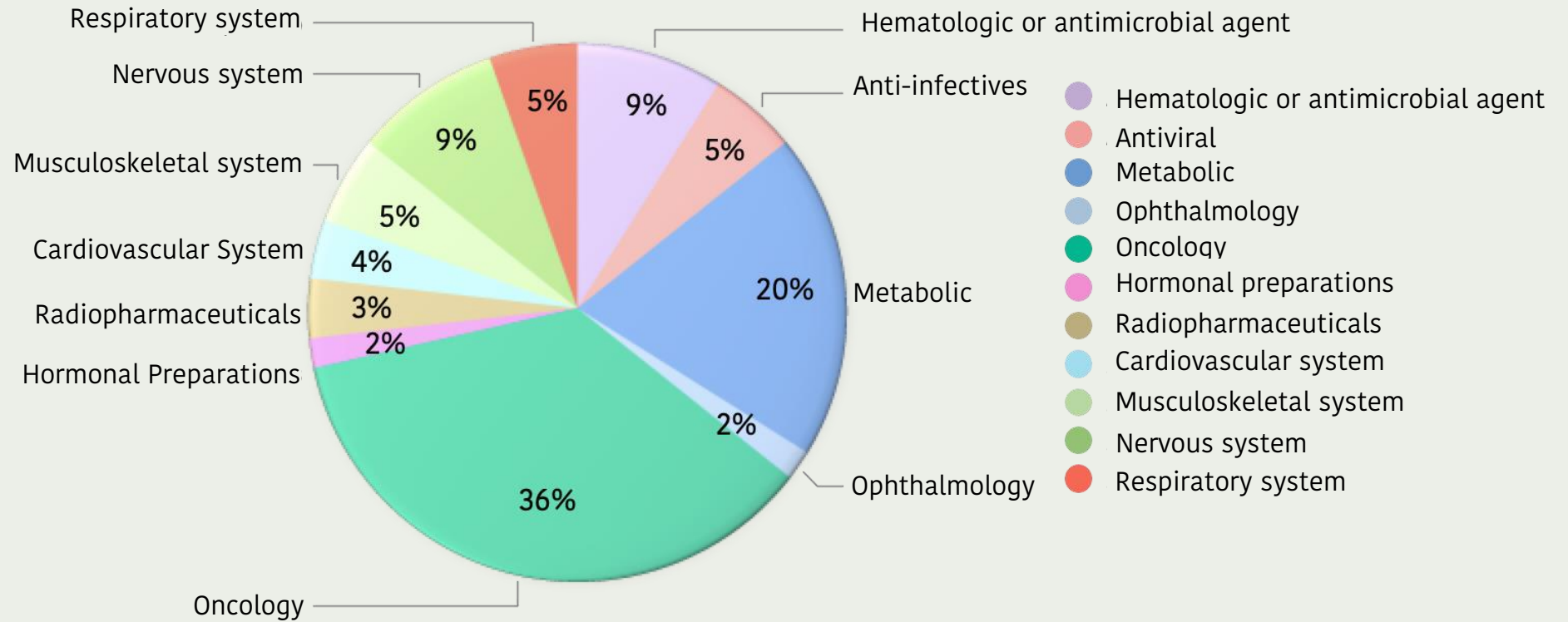


In 2021, **50%** of the ODs that have reached Spain are currently NHS funded, four points more than in 2020 (46%).

# Evolution of national indicators and average waiting time for funding 2019-2021.



## Drugs financed by the NHS by therapeutic area





# Orphan drugs funded in 2021



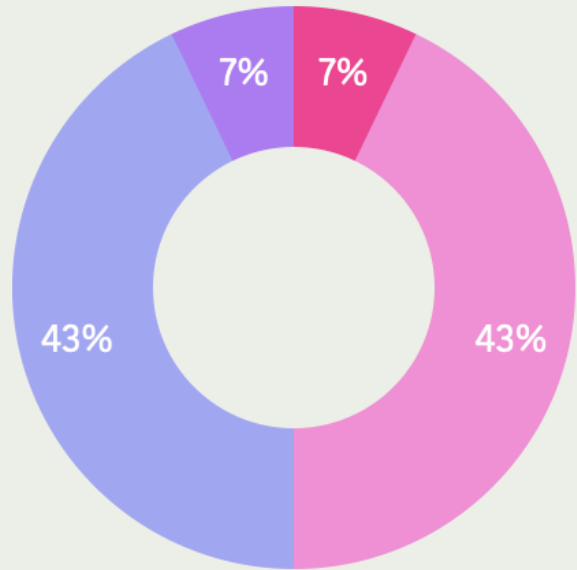


## Orphan drugs funded by the NHS in 2021

Commercial Name	Laboratoy	NC date	NHS funding date	Months from NC to funding
<b>1. CRYSVITA</b>	Kyowa Kirin	11/05/2018	01/01/2021	32
<b>2. TAKHZYRO</b>	Takeda	28/03/2019	01/03/2021	23
<b>3. LUXTURNA</b>	Novartis	02/04/2019	01/05/2021	25
<b>4. CABLIVI</b>	Ablynx/Sanofi	25/09/2018	01/07/2021	34
<b>5. MEPSEVII</b>	Ultragenyx	19/09/2018	01/07/2021	34
<b>6. POTELIGEO</b>	Kyowa Kirin	05/06/2019	01/07/2021	25
<b>7. PREVYMIS</b>	MSD	26/02/2018	01/08/2021	42
<b>8. TREPULMIX</b>	SciPharm/AOP Orphan	07/04/2021	01/08/2021	4
<b>9. EPIDYOLEX</b>	Gw Pharma	05/12/2019	01/09/2021	21
<b>10. GIVLAARI</b>	Alnylam	28/04/2020	01/09/2021	16
<b>11. POLIVY</b>	Roche	18/02/2020	01/09/2021	19
<b>12. QUENODESOXYCHOLIC ACID</b>	Leadiant	21/06/2019	01/12/2021	30
<b>13. KAFTRIO</b>	Vertex	23/10/2020	01/12/2021	13
<b>14. ZOLGENSMA</b>	Novartis (Avexis)	28/07/2020	01/12/2021	16

# Waiting time and funding conditions

Percentage of products funded in 2021 by waiting years



- Less than 1 year
- Between 1 and 2 years
- Between 2 and 3 years
- More than 3 years

- 67% They have been through the CIPM two or more times.
- 100% Approved with funding conditions:
  - 43% with 3 or more funding conditions
  - 93% Monitoring
  - 50% Expenditure ceiling or maximum cost per patient
  - 21% Payment by results/risk sharing
- 43% Restricted funding in Spain

# Detailed funding criteria and conditions ODs 2021

Commercial Name	Laboratoy	NHS funding date	CIPM Meetings	Criteria for funding <sup>1</sup>	Funding conditions
<b>1. CRYSVITA</b>	Kyowa Kirin	01/01/2021	November 14, 2020	a) y c)	<ul style="list-style-type: none"> <li>• Payment by results/Risk sharing</li> <li>• Maximum annual treatment cost per patient</li> <li>• Pharmacoclinical protocol</li> <li>• Follow-up</li> </ul>
<b>2. TAKHZYRO</b>	Takeda	01/03/2021	December 17, 2020	a) y c)	<ul style="list-style-type: none"> <li>• Tracking</li> </ul>
<b>3. LUXTURNA</b>	Novartis	01/05/2021	March 3, 2021 December 17, 2020	a) y c)	<ul style="list-style-type: none"> <li>• Pharmacoclinical protocol</li> <li>• Tracking</li> <li>• Expenditure ceiling</li> </ul>
<b>4. CABLIVI</b>	Ablynx/Sanofi	01/07/2021	May 6, 2021 December 17, 2020	a) y c)	<ul style="list-style-type: none"> <li>• Tracking</li> </ul>
<b>5. MEPSEVII</b>	Ultragenyx	01/07/2021	May 6, 2021 September 15, 2020	a) y c)	<ul style="list-style-type: none"> <li>• Maximum cost per patient</li> <li>• Pharmacoclinical protocol</li> <li>• Tracking</li> </ul>
<b>6. POTELIGEO</b>	Kyowa Kirin	01/07/2021	May 6, 2021 March 3, 2021	a) y c)	<ul style="list-style-type: none"> <li>• Tracking</li> <li>• Expenditure ceiling</li> </ul>
<b>7. PREVYMIS</b>	MSD	01/08/2021	June 10, 2021 April 7, 2021 February 3, 2021 April 25, 2019 February 8, 2019	a) y c)	<ul style="list-style-type: none"> <li>• Tracking</li> <li>• Expenditure ceiling</li> </ul>
<b>8. TREPULMIX</b>	SciPharm/AOP Orphan	01/08/2021	June 10, 2021	a) y c)	<ul style="list-style-type: none"> <li>• Annual review of sales and prices</li> </ul>
<b>9. EPIDYOLEX</b>	Gw Pharma	01/09/2021	July 08, 2021	a) y c)	<ul style="list-style-type: none"> <li>• Tracking</li> <li>• Expenditure ceiling</li> </ul>
<b>10. GIVLAARI</b>	Alnylam	01/09/2021	July 08, 2021 May 06, 2021	a) y c)	<ul style="list-style-type: none"> <li>• Maximum cost per patient</li> <li>• Tracking</li> </ul>
<b>11. POLIVY</b>	Roche	01/09/2021	July 08, 2021 February 3, 2021	a) y c)	<ul style="list-style-type: none"> <li>• Pharmacoclinical protocol</li> <li>• Shared risk/Payment by results</li> <li>• Tracking</li> </ul>
<b>12. QUENODESOXYCHOLIC ACID</b>	Leadiant	01/12/2021	September 30, 2021 July 08, 2021	a) y c)	<ul style="list-style-type: none"> <li>• Tracking</li> </ul>
<b>13. KAFTRIO</b>	Vertex	01/12/2021	October 28, 2021	a) y c)	<ul style="list-style-type: none"> <li>• Price/volume agreement</li> <li>• Pharmacoclinical protocol</li> <li>• Tracking</li> <li>• Supply at lower cost</li> </ul>
<b>14. ZOLGENSMA</b>	Novartis (Avexis)	01/12/2021	October 28, 2021 April 7, 2021	a) y c)	<ul style="list-style-type: none"> <li>• Price/volume agreement</li> <li>• Pharmacoclinical protocol</li> <li>• Payment by results/ Risk sharing</li> <li>• Tracking</li> </ul>

## <sup>1</sup>Criteria for funding:

a) Severity, duration and consequences of the different pathologies for which they are indicated.

c) Therapeutic and social value of the drug and incremental clinical benefit, taking into account its cost-effectiveness.

Source: [Interministerial Commission on Drug Prices Agreements](#)

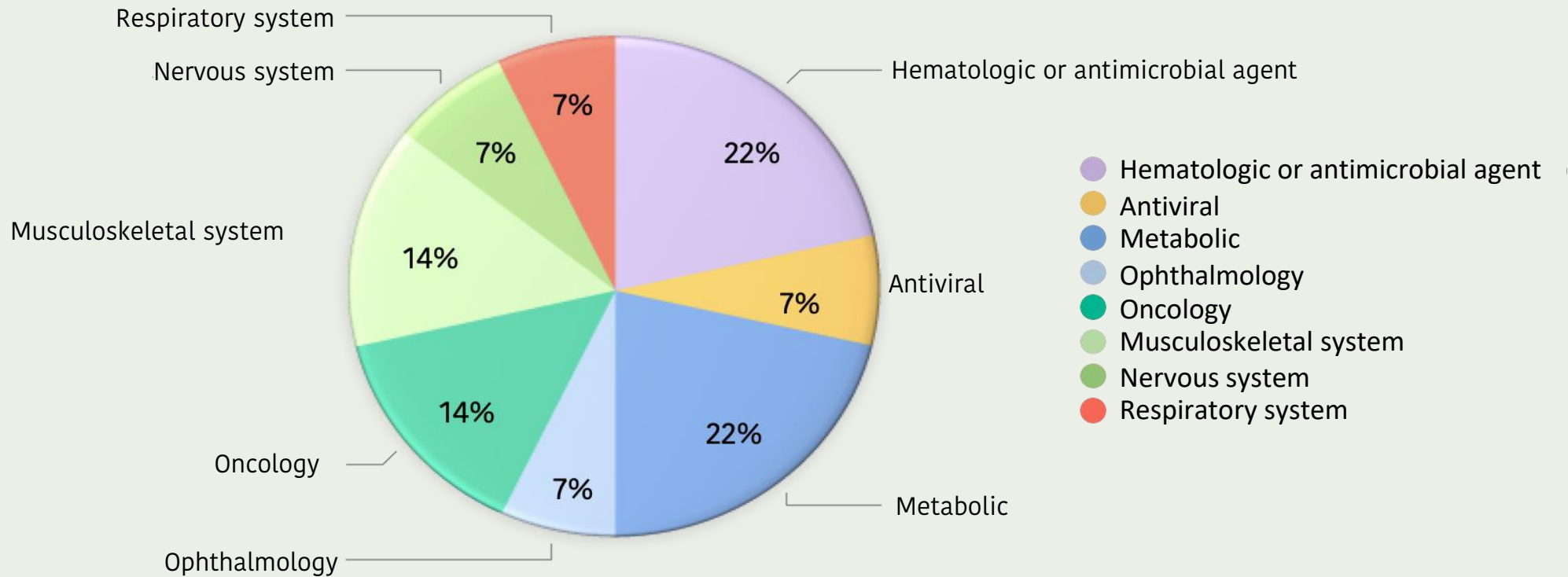
# Detail of funded indications during 2021

Commercial Name	Laboratoy	Funding status	Funded indications (Ministry of Health)	EMA indication
<b>1. CRYSVITA</b>	Kyowa Kirin	Yes for certain indications	It is indicated for the treatment of X-linked hypophosphatemia with radiographic signs of bone disease in children 1 year and older and adolescents with a growing skeleton.X-linked hypophosphatemia with radiographic signs of bone disease in children 1 year and older and adolescents with a growing skeleton.The drug is financed through a pay-for-results agreement with specific clinical conditions (see conditions in the Nomenclator).Translated with www.DeepL.com/Translator (free version)	<ul style="list-style-type: none"> <li>• Crysvita is indicated for the treatment of X-linked hypophosphataemia, in children and adolescents aged 1 to 17 years with radiographic evidence of bone disease, and in adults.</li> </ul>
<b>2. TAKHZYRO</b>	Takeda	Yes	Routine prevention of recurrent attacks of hereditary angioedema (HAE) in patients 12 years of age and older.	<ul style="list-style-type: none"> <li>• Coinciding</li> </ul>
<b>3. LUXTURNA</b>	Novartis	Yes	Treatment of adults and children with vision loss due to confirmed biallelic RPE65 mutation-associated retinal dystrophy who have sufficient viable retinal cells.	<ul style="list-style-type: none"> <li>• Coinciding</li> </ul>
<b>4. CABLIVI</b>	Ablynx/Sanofi	Yes	Treatment of adults and adolescents aged 12 years and older weighing at least 40 kg who present with an episode of acquired thrombotic thrombocytopenic purpura (aTTP) together with plasma exchange and immunosuppression who have an insufficient response to standard therapy or who suffer recurrences.	<ul style="list-style-type: none"> <li>• Coinciding</li> </ul>
<b>5. MEPSEVII</b>	Ultragenyx	Yes	Treatment of non-neurological manifestations of mucopolysaccharidosis VII (MPS VII; Sly syndrome) in patients diagnosed with mucopolysaccharidosis VII or Sly disease by means of: - enzymatic study of beta-glucuronidase deficiency, or -by genetic study with pathologic mutations of this gene, or -by both simultaneously.	<ul style="list-style-type: none"> <li>• Coinciding</li> </ul>
<b>6. POTELIGEO</b>	Kyowa Kirin	Yes for certain indications	Treatment of patients with Sézary syndrome who are not candidates for allogeneic hematopoietic stem cell transplantation (HSCT) and with CCR4 skin expression greater than or equal to 10%, if determination is available.	<ul style="list-style-type: none"> <li>• Poteligeo is indicated for the treatment of adult patients with mycosis fungoides (MF) or Sézary syndrome (SS) who have received at least one prior systemic therapy.</li> </ul>

# Detail of funded indications during 2021

Commercial Name	Laboratory	Funding status	Funded indications	EMA indication
<b>7. PREVYMIS</b>	MSD	Yes	Prophylaxis of cytomegalovirus (CMV) reactivation and CMV disease in adult seropositive allogeneic hematopoietic stem cell transplant (HSCT) recipients at high risk of reactivation. (Nomeclator Eligible Patient Conditions)	<ul style="list-style-type: none"> <li>• Coinciding</li> </ul>
<b>8. TREPULMIX</b>	SciPharm/AOP Orphan	Yes	Treatment of adult patients with WHO functional class (FC) III or IV and a) with inoperable chronic thromboembolic pulmonary hypertension (CTEPH) or b) persistent or recurrent CTEPH after surgical treatment to improve exercise capacity.	<ul style="list-style-type: none"> <li>• Coinciding</li> </ul>
<b>9. EPIDYOLEX</b>	Gw Pharma	Yes for certain indications	As adjunctive treatment of seizures associated with Lennox-Gastaut syndrome (LGS) or Dravet syndrome (DS) in conjunction with clobazam, for patients as young as two years old, who have not responded to treatment with 3 antiepileptic drugs.	<ul style="list-style-type: none"> <li>• Use as adjunctive therapy of seizures associated with Lennox Gastaut syndrome (LGS) or Dravet syndrome (DS), in conjunction with clobazam, for patients 2 years of age and older.</li> </ul>
<b>10. GIVLAARI</b>	Alnylam	Yes for certain indications	Treatment of acute intermittent hepatic porphyria in adult patients who must meet both of the following criteria: <ol style="list-style-type: none"> <li>Patient with &gt; or equal 2 crises in the last 6 months.</li> <li>Patient with urine ALA or PBG levels &gt; or equal to 4 times the upper limit of normal range [ULN] and chronic symptoms. In the case of patients on chronic treatment with hemin, they must meet the condition of patient with ALA or PBG levels &gt; or equal to 4 times the upper limit of the normal range [ULN] and chronic symptoms and/or history of &gt; or equal to 4 crises per year.</li> </ol> Translated with www.DeepL.com/Translator (free version)	<ul style="list-style-type: none"> <li>• Treatment of acute hepatic porphyria (AHP) in adults and adolescents aged 12 years and older.</li> </ul>
<b>11. POLIVY</b>	Roche	Yes	Polivy in combination with bendamustine and rituximab is indicated for the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) who are not candidates for hematopoietic stem cell transplantation..	<ul style="list-style-type: none"> <li>• Coinciding</li> </ul>
<b>12. QUENODESOXYCHOLIC ACID</b>	Leadiant	Yes	Treatment of inborn errors in primary bile acid synthesis caused by 27-sterol-hydroxylase deficiency (manifesting as cerebrotendinous xanthomatosis (XCT)) in infants, children and adolescents from 1 month to 18 years of age, and adults.	<ul style="list-style-type: none"> <li>• Coinciding</li> </ul>
<b>13. KAFTRIO</b>	Vertex	Yes	Kaftrio is indicated in a combination administration regimen with ivacaftor 150 mg tablets for the treatment of cystic fibrosis (CF) in patients 12 years of age or older with at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.	<ul style="list-style-type: none"> <li>• In a combination regimen with ivacaftor for the treatment of cystic fibrosis (CF) in patients aged 6 years and older who have at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.</li> </ul>
<b>14. ZOLGENSMA</b>	Novartis (Avexis)	Yes for certain indications	Treatment of patients with spinal muscular atrophy (SMA) on 5q with a biallelic mutation in the SMN1 gene and a clinical diagnosis of SMA type 1, as well as for patients with presymptomatic SMA on 5q with a biallelic mutation in the SMN1 gene who meet the clinical criteria of the pay-for-results agreement (available from the Nomeclator).	<ul style="list-style-type: none"> <li>• Treatment of:                             <ol style="list-style-type: none"> <li>1) patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA Type 1, or</li> <li>2) patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene.</li> </ol> </li> </ul>

# Orphan drugs funded during 2021 by therapeutic area



# Orphan drugs funded in 2021, situation in Europe

Orphan drugs funded in 2021



Commercial Name	Laboratoy	NHS funding date	Other European countries where it has obtained a favorable funding opinion
<b>1. CRYSVITA</b>	Kyowa Kirin	01/01/2021	<ol style="list-style-type: none"> <li>1. Germany</li> <li>2. Belgium</li> <li>3. Denmark</li> <li>4. Slovakia</li> <li>5. Slovenia</li> <li>6. Finland</li> <li>7. France</li> <li>8. Italy</li> <li>9. Luxembourg</li> <li>10. Norway</li> <li>11. Romania</li> <li>12. Sweden</li> <li>13. UK</li> </ol>
<b>2. TAKHZYRO</b>	Takeda	01/03/2021	<ol style="list-style-type: none"> <li>1. Bulgaria</li> <li>2. Denmark</li> <li>3. Slovakia</li> <li>4. Slovenia</li> <li>5. Finland</li> <li>6. France</li> <li>7. Germany</li> <li>8. Ireland</li> <li>9. Italy</li> <li>10. Liechtenstain</li> <li>11. Norway</li> <li>12. Poland</li> <li>13. Switzerland</li> <li>14. UK</li> </ol>
<b>3. LUXTURNA</b>	Novartis	01/05/2021	<ol style="list-style-type: none"> <li>1. Belgium</li> <li>2. Croatia</li> <li>3. Denmark</li> <li>4. France</li> <li>5. Germany</li> <li>6. Ireland</li> <li>7. Italy</li> <li>8. Netherlands</li> <li>9. UK</li> </ol>
<b>4. CABLIVI</b>	Ablynx/Sanofi	01/07/2021	<ol style="list-style-type: none"> <li>1. Belgium</li> <li>2. Slovakia</li> <li>3. Slovenia</li> <li>4. France</li> <li>5. Germany</li> <li>6. Netherlands</li> <li>7. Italy</li> <li>8. Luxembourg</li> <li>9. Czech Republic</li> <li>10. Romania</li> <li>11. UK</li> </ol>

Detailed information on countries and funding dates in annexes

Note: Data provided by Picentric ONE by Eversana™ (31/12/2021). *Global Pricing & Market Access* data. More information can be found [here](#)

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Commercial Name	Laboratoy	NHS funding date	Other European countries where it has obtained a favorable funding opinion
<b>5. MEPSEVII</b>	Ultragenyx	01/07/2021	1. Germany 2. Italy
<b>6. POTEFIGEO</b>	Kyowa Kirin	01/07/2021	1. Belgium 2. Slovenia 3. France 4. Germany 5. Italy 6. UK
<b>7. PREVYMIS</b>	MSD	01/08/2021	1. Belgium 2. Croatia 3. Denmark 4. Slovenia 5. Finland 6. France 7. Germany 8. Hungary 9. Italy 10. Ireland 11. Netherlands 12. Latvia 13. Liechtenstein 14. Luxembourg 15. Norway 16. Portugal 17. Czech Republic 18. Romania 19. Sweden 20. Switzerland 21. UK
<b>8. TREPULMIX</b>	SciPharm/AOP Orphan	01/08/2021	1. Germany 2. Latvia 3. Romania
<b>9. EPIDYOLEX</b>	Gw Pharma	01/09/2021	1. Germany 2. Austria 3. Denmark 4. Finland 5. Italy 6. Norway 7. Sweden 8. UK

Detailed information on countries and funding dates in annexes

Note: Data provided by Picentric ONE by Eversana™ (31/12/2021). *Global Pricing & Market Access* data. More information can be found [here](#)

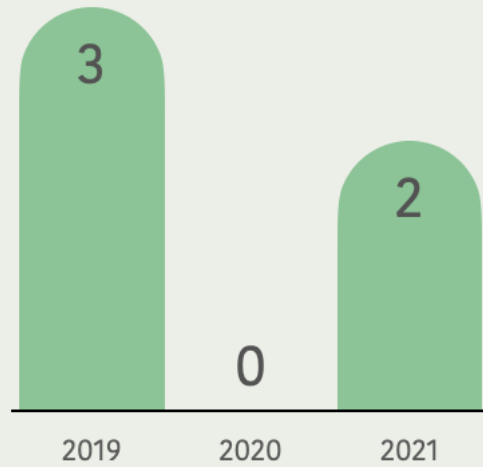
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Commercial Name	Laboratoy	NHS funding date	Other European countries where it has obtained a favorable funding opinion
<b>10. GIVLAARI</b>	Alnylam	01/09/2021	<ol style="list-style-type: none"> <li>1. Belgium</li> <li>2. Slovenia</li> <li>3. France</li> <li>4. Germany</li> <li>5. Italy</li> <li>6. Luxembourg</li> <li>7. Netherlands</li> </ol>
<b>11. POLIVY</b>	Roche	01/09/2021	<ol style="list-style-type: none"> <li>1. Germany</li> <li>2. Bulgaria</li> <li>3. Slovenia</li> <li>4. Ireland</li> <li>5. Latvia</li> <li>6. Czech Republic</li> <li>7. UK</li> </ol>
<b>12. QUENODESOXYCHOLIC ACID</b>	Leadiant	01/12/2021	<ol style="list-style-type: none"> <li>1. Germany</li> <li>2. France</li> <li>3. Netherlands</li> <li>4. Italy</li> <li>5. Norway</li> <li>6. UK</li> </ol>
<b>13. KAFTRIO</b>	Vertex	01/12/2021	<ol style="list-style-type: none"> <li>1. Bulgaria</li> <li>2. Croatia</li> <li>3. Slovakia</li> <li>4. Slovenia</li> <li>5. Finland</li> <li>6. France</li> <li>7. Germany</li> <li>8. Italy</li> <li>9. Ireland</li> <li>10. Luxembourg</li> <li>11. Norway</li> <li>12. Portugal</li> <li>13. UK</li> </ol>
<b>14. ZOLGENSMA</b>	Novartis (Avexis)	01/12/2021	<ol style="list-style-type: none"> <li>1. Germany</li> <li>2. Belgium</li> <li>3. Croatia</li> <li>4. Denmark</li> <li>5. Finland</li> <li>6. Slovenia</li> <li>7. Ireland</li> <li>8. Italy</li> <li>9. Latvia</li> <li>10. Portugal</li> <li>11. Czech Republic</li> <li>12. Romania</li> <li>13. UK</li> </ol>

Detailed information on countries and funding dates in annexes

# Advanced therapies with orphan designation

# Advanced therapies with orphan designation funded in Spain 2019-2021.



Commercial Name	Laboratoy	Indication	NC date	NHS funding date	Months from NC to funding	CIPM	Funding conditions
LUXTURNA	Novartis	Retinal dystrophy	02/04/2019	01/05/2021	25	March 3, 2021	<ul style="list-style-type: none"> <li>• Expenditure ceiling</li> <li>• Pharmacoclinical protocol</li> <li>• Tracking</li> </ul>
ZOLGENSMA	Novartis (Avexis)	Spinal muscular atrophy	28/07/2020	01/12/2021	16	October 28, 2021 April 7, 2021	<ul style="list-style-type: none"> <li>• Price/volume agreement</li> <li>• Pharmacoclinical protocol</li> <li>• Shared risk/Payment by results</li> <li>• Tracking</li> </ul>

# Advanced therapies with orphan designation funded in Spain 2019-2021, situation in Europe.

Countries in which they have obtained a favorable funding opinion prior to Spain

LUXTURNA\*



ZOLGENSMA\*\*



\*Funding dates for Belgium and Croatia are not available.

\*\*Funding dates for Belgium, Croatia, Slovenia, Latvia and Czech Republic are not available.

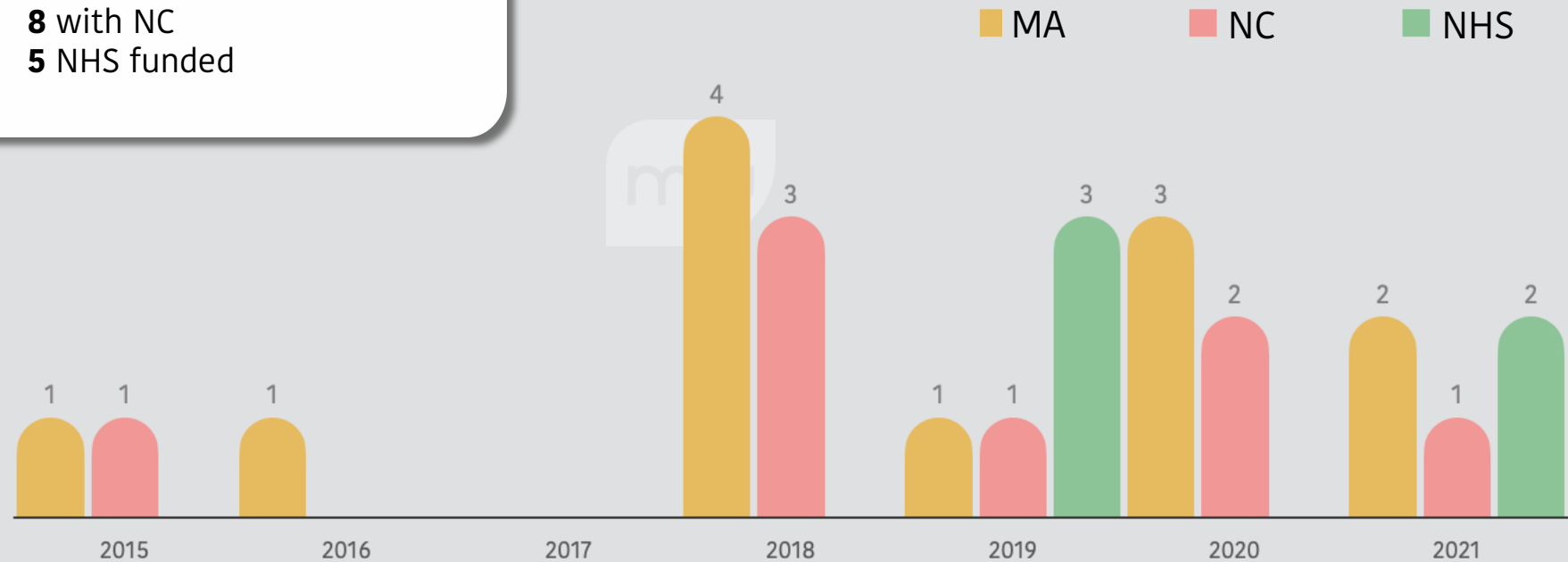
# Advanced therapies with orphan designation EU and Spain 2015-2021

ODs funded in 2021



At December 31, 2021:

- **12** advanced therapies with MA
- **8** with NC
- **5** NHS funded





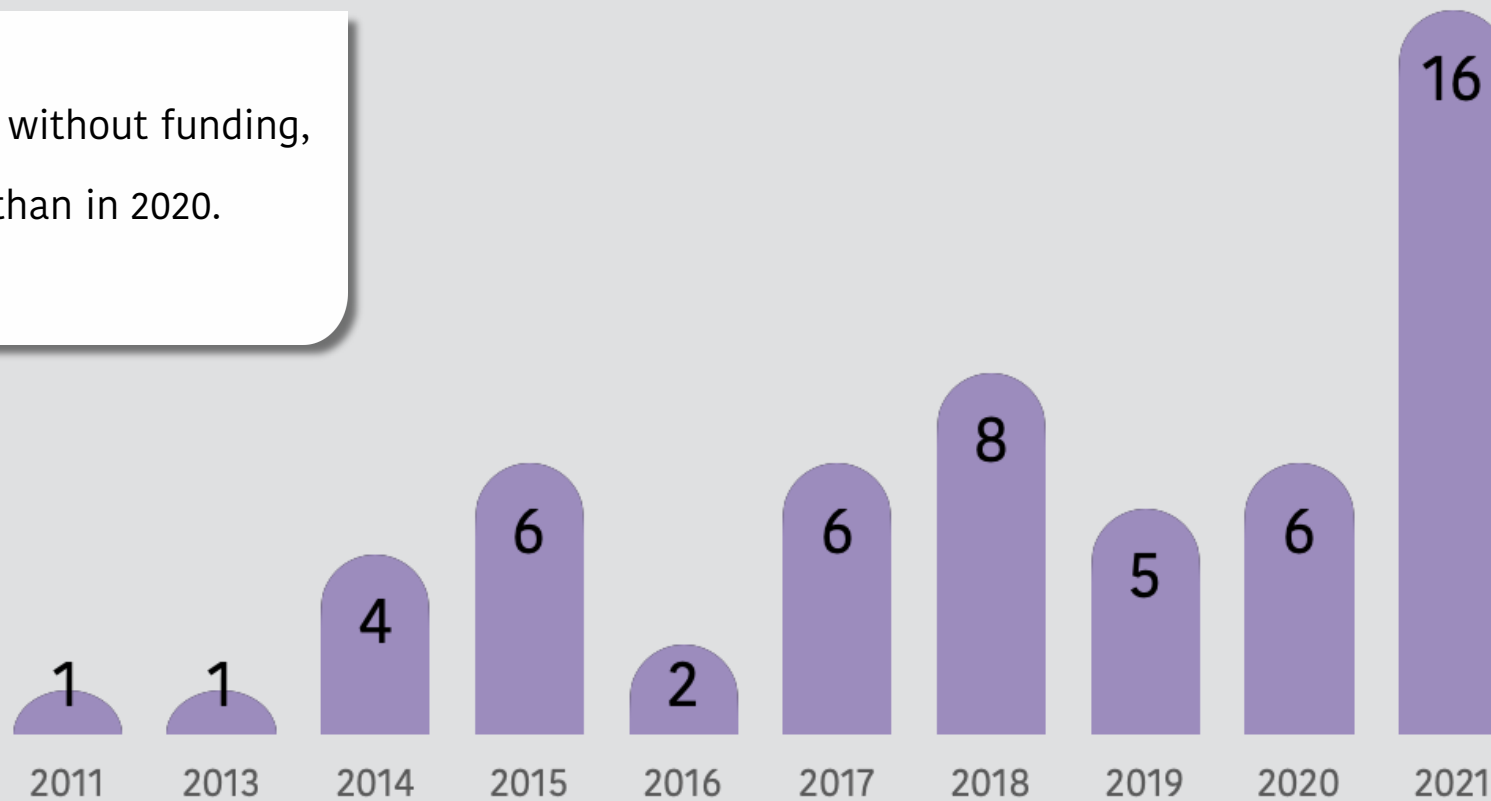
# Orphan Drugs not funded





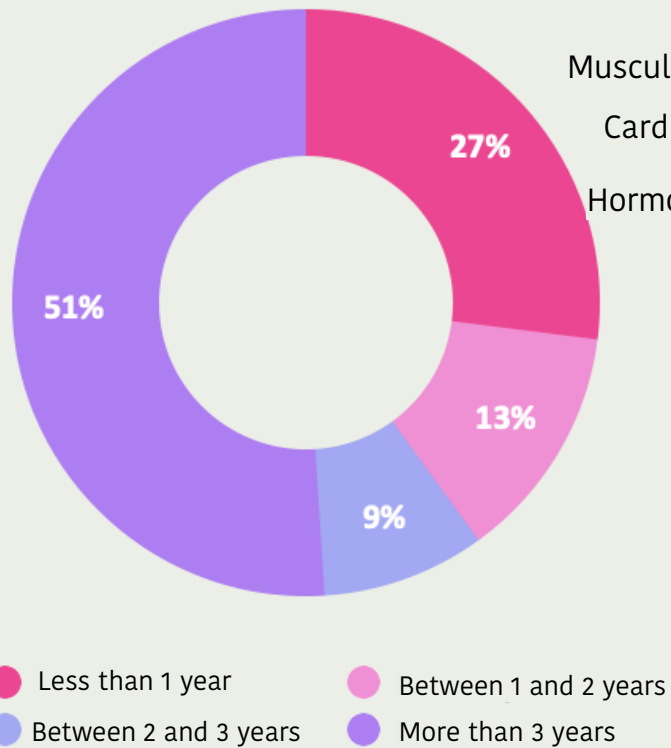
# Number of orphan drugs without funding in Spain by year of NC

**55** ODs without funding,  
**2** more than in 2020.

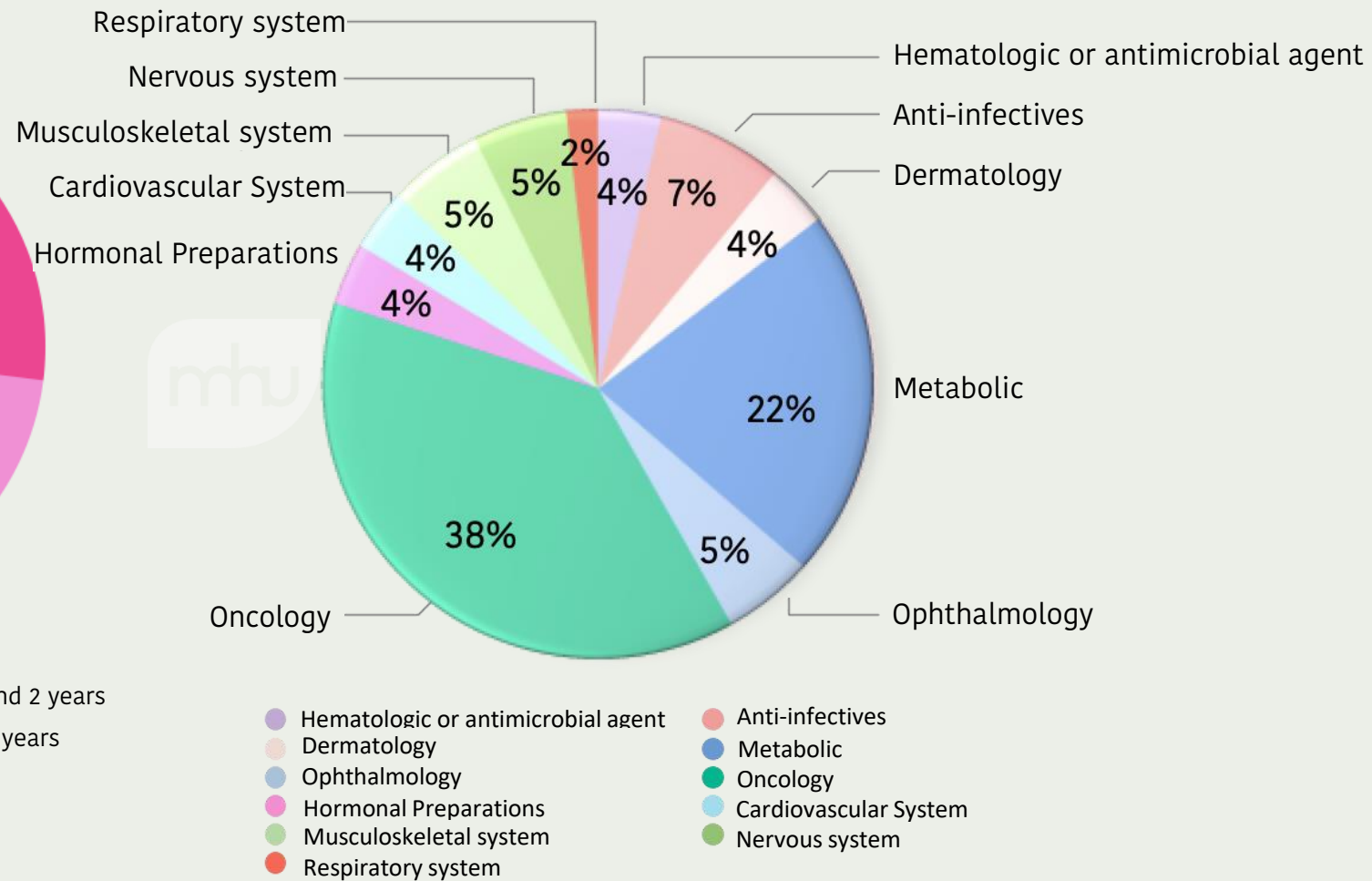




### ODs by years of waiting



### ODs by therapeutic area



# ODs with NC in 2021



	Name	Laboratoy	NC date	Funding date	Months NC-Funding	Funding*	Months from NC	Funding status	CIPM (Unfavorable resolution before funding)
1	TREPULMIX	SciPharm/AOP Orphan	07/04/2021	01/08/2021	4			Funded	
2	<b>AMGLIDIA</b>	Ammtek	14/01/2021			01/12/2021	12	Not funded	30/09/2021 08/07/2021
3	<b>ADAKVEO</b>	Novartis	01/02/2021				11	Not funded	
4	<b>TRECONDI</b>	Medac GMBH	03/02/2021				11	Not funded	
5	<b>ISTURISA</b>	Recordati Rare Diseases	12/02/2021				11	Not funded	25/11/2021
6	<b>INREBIC</b>	BMS	26/02/2021				10	Not funded	
7	<b>EVRYSDI</b>	Roche	21/04/2021				8	Not funded	
8	<b>ENSPRYNG</b>	Roche	26/07/2021				5	Not funded	
9	<b>FINTEPLA</b>	Zogenix/MSD	02/06/2021				7	Not funded	
10	<b>HEPCLUDEX</b>	Gilead	16/10/2021				2	Not funded	
11	<b>KOSELUGO</b>	AstraZeneca	27/07/2021				5	Not funded	
12	<b>PEMAZYRE</b>	Incyte	07/05/2021				8	Not funded	25/11/2021
13	<b>LIBMELDY</b>	Orchard Therapeutics	19/10/2021				2	Not funded	
14	<b>BYLVAY</b>	Albireo	21/12/2021				0	Not funded	
15	<b>MINJUVI</b>	Incyte	28/09/2021				3	Not funded	
16	<b>ASPAVELI</b>	SOBI	30/12/2021				0	Not funded	
17	<b>IDEFIRIX</b>	Hansa	09/12/2021				1	Not funded	



\* The date of non-funding indicated in the Nomenclátor is the first date of non-funding/exclusion.

# ODs with NC in 2020



	Name	Laboratoy	NC date	Funding date	Months NC-Funding	Months from NC	Funding status	CIPM (Unfavorable resolution before funding)
1	POLIVY	Roche	18/02/2020	01/09/2021	19		Funded	03/02/2021
2	GIVLAARI	Alnylam	28/04/2020	01/09/2021	16		Funded	
3	KAFTRIO	Vertex	23/10/2020	01/12/2021	13		Funded	
4	ZOLGENSMA	Novartis (Avexis)	28/07/2020	01/12/2021	16		Funded	
5	<b>WAYLIVRA</b>	Akcea (SOBI)	02/03/2020			22	Not funded	30/09/2021
6	<b>DAURISMO</b>	Pfizer	24/07/2020			18	Not funded	16/12/2021
7	<b>REBLOZYL</b>	BMS	18/08/2020			17	Not funded	
8	<b>BLENREP</b>	Glaxosmithkline	23/09/2020			15	Not funded	16/12/2021
9	<b>TECARTUS</b>	Kite Pharma	28/12/2020			12	Not funded	16/12/2021
10	<b>OXLUMO</b>	Alnylam	30/12/2020			12	Not funded	

## ODs with NC in 2019

	Name	Laboratoy	NC date	Funding date	Months NC-Funding	Date of non-funding/exclusion*	Months from NC	Funding status	CIPM (Unfavorable resolution before funding)
1	SYMKEVI	Vertex	17/01/2019	1/11/2019	10			Funded	
2	TEGSEDI	Akcea (SOBI)	09/09/2019	1/05/2020	8			Funded	
3	TAKHZYRO	Takeda	28/03/2019	01/03/2021	23			Funded	
4	LUXTURNA	Novartis	02/04/2019	01/05/2021	25			Funded	
5	POTELIGEO	Kyowa Kirin	05/06/2019	01/07/2021	25			Funded	03/03/2021
6	EPIDYOLEX	GW Pharma	05/12/2019	01/09/2021	21			Funded	
7	ÁCIDO QUENODES-OXICÓLICO	Leadiant	21/06/2019	01/12/2021	30			Funded	14/10/2020
8	<b>VERKAZIA</b>	Santen	08/04/2019			01/07/2021	33	Not funded	07/04/2021
9	<b>NAMUSCLA</b>	Lupin/Exeltis	30/04/2019			01/06/2021	33	Not funded	07/04/2021 17/12/2020
10	<b>BRINEURA</b>	Biomarin	21/06/2019				31	Not funded	
11	<b>XOSPATA</b>	Astellas	05/12/2019			01/06/2021	25	Not funded	07/04/2021 03/02/2021
12	<b>PALYNZIQ</b>	Biomarin	16/12/2019			01/10/2021	25	Not funded	10/06/2021

\*The date of non-funding indicated in the Nomenclátor is the first date of non-funding/exclusion.

# ODs with NC in 2018



	Name	Laboratory	NC date	Funding date	Months NC-Funding	Date of non-funding/exclusion*	Months from NC	Funding status	CIPM (Unfavorable resolution before funding)
1	CRYSVITA	Kyowa Kirin	11/05/2018	01/01/2021	32			Funded	
2	CABLIVI	Ablynx/Sanofi	25/09/2018	01/07/2021	34			Funded	17/12/2020
3	MEPSEVII	Ultragenyx	19/09/2018	01/07/2021	34			Funded	15/09/2020
4	PREVYMIS	MSD	26/02/2018	01/08/2021	42			Funded	25/04/2019 08/02/2019
5	ONPATTRO	Alnylam	31/10/2018	01/05/2020	18			Funded	
6	ZEJULA	Glaxosmithkline	08/03/2018	01/08/2019	17			Funded	19/03/2019
7	ALOFISEL	Takeda	19/07/2018	01/09/2019	14			Funded	
8	MYLOTARG	Pfizer	25/05/2018	01/07/2019	13			Funded	
9	YESCARTA	Kite Pharma	06/09/2018	01/07/2019	10			Funded	14/11/2018
10	KYMRIAH	Novartis	17/09/2018	01/01/2019	4			Funded	14/11/2018
11	<b>NATPAR</b>	Takeda	12/02/2018			01/02/2019	47	Not funded	
12	<b>JORVEZA</b>	Dr. Falk Pharma	07/03/2018			01/09/2019	47	Not funded	30/05/2019 19/03/2019
13	<b>PROCYSBI</b>	Chiesi	31/05/2018			01/04/2019	44	Not funded	08/02/2019
14	<b>RAXONE</b>	Chiesi	29/06/2018			01/09/2021	43	Not funded	08/07/2021 07/04/2021
15	<b>QARZIBA</b>	Eusa Pharma	06/09/2018				40	Not funded	
16	<b>LAMZEDE</b>	Chiesi	07/09/2018			01/07/2021	40	Not funded	06/05/2021 14/10/2020
17	<b>MYALEPTA</b>	Amryt	06/11/2018			01/08/2020	38	Not funded	25/11/2021 10/06/2021 17/06/2020 28/11/2019
18	<b>VYXEOS</b>	Jazz Pharma	19/12/2018			01/12/2020	37	Not funded	28/10/2021 14/10/2020 28/11/2019

\* The date of non-funding indicated in the Nomenclátor is the first date of non-funding/exclusion.

# ODs with NC in 2017



	Name	Laboratoy	NC date	Funding date	Months NC-Funding	Date of non-funding/exclusion*	Months from NC	Funding status	CIPM** (Unfavorable resolution before funding)
1	BESPONSA	Pfizer	21/07/2017	01/07/2019	24			Funded	08/02/2019
2	RYDAPT	Novartis	30/10/2017	01/04/2019	17			Funded	
3	LUTATHERA	Advanced Accelerator Applications	14/11/2017	01/04/2019	17			Funded	16/07/2018
4	SOMAKIT	Advanced Accelerator Applications	01/03/2017	01/04/2018	13			Funded	
5	OCALIVA	Intercept	31/01/2017	01/03/2018	13			Funded	
6	DELTYBA	Otsuka	05/05/2017	01/02/2018	9			Funded	
7	SPINRAZA	Biogen	06/07/2017	01/03/2018	8			Funded	
8	RAVICTI	Immedica/SOBI	19/06/2017	01/12/2017	6			Funded	
9	NINLARO	Takeda	06/02/2017			01/03/2019	60	Not funded	07/04/2021 03/02/2021 08/02/2019 31/10/2018 22/03/2018
10	SCENESSE	Clinuvel	27/03/2017			01/05/2018	58	Not funded	
11	LEDAGA	Helsinn (Recordati Rare Diseases)	18/04/2017			01/04/2018	57	Not funded	07/04/2021 03/02/2021
12	OXERVATE	Dompe	02/10/2017			01/07/2019	52	Not funded	30/11/2018
13	CYSTADROPS	Recordati Rare Diseases	27/12/2017			01/12/2018	49	Not funded	25/05/2018 31/10/2018
14	XERMELO	Ipsen	22/12/2017			01/06/2018	49	Not funded	

\*The date of non-funding indicated in the Nomenclátor is the first date of non-funding/exclusion.

\*\*Agreements previous November 2017 are not available.

# ODs with NC in 2016



	Name	Laboratoy	NC date	Funding date	Months NC-Funding	Date of non-funding/exclusion*	Months from NC	Funding status	CIPM** (Unfavorable resolution before funding)
1	WAKIX	Bioprojet	03/08/2016	01/11/2020	52			Funded	
2	ALPROLIX	SOBI	23/06/2016	01/03/2020	45			Funded	
3	IDELVION	Csl Behring	30/08/2016	01/03/2020	43			Funded	
4	KANUMA	Alexion	08/01/2016	01/11/2017	22			Funded	
5	GALAFOLD	Amicus	29/08/2016	01/02/2018	17			Funded	
6	CERDELGA	Genzyme	02/02/2016	01/01/2017	11			Funded	
7	ICLUSIG	Incyte	13/09/2016	01/07/2017	10			Funded	
8	CRESEMBA	Basilea/Pfizer	15/02/2016	01/11/2016	9			Funded	
9	DARZALEX	Janssen	23/06/2016	01/12/2016	5			Funded	
10	<b>BRONCHITOL</b>	Pharmaxis (Chiesi)	15/07/2016			01/05/2017	67	Not funded	
11	<b>ONIVYDE</b>	Servier	11/11/2016			01/12/2019	63	Not funded	6/11/2017 25/5/2018

\*The date of non-funding indicated in the Nomenclátor is the first date of non-funding/exclusion.

\*\*Agreements previous November 2017 are not available.



# Unfunded ODs with NC between 2011 and 2015



	Commercial Name	Laboratoy	NC date	Date of non-funding/exclusion*	Months from NC	CIPM** (Unfavorable resolution before funding)
1	<b>TOBI</b>	Mylan	12/08/2011	01/12/2012	126	
2	<b>XALUPRINE</b>	Nova Laboratories	05/02/2013	ND	108	
3	<b>GRANUPAS</b>	Eurocept	13/06/2014	01/07/2016	92	
4	<b>SIRTURO</b>	Janssen	18/09/2014	01/07/2016	89	
5	<b>NEXOBRID</b>	Mediwound	15/09/2014	01/11/2015	89	
6	<b>TRANSLARNA</b>	Ptc Therapeutics	01/12/2014	01/04/2016	86	30/10/2019 25/04/2019 08/02/2019
7	<b>STRENSIQ</b>	Alexion	15/10/2015	01/07/2018	76	25/05/2018 22/03/2018
8	<b>BLINCYTO</b>	Amgen	16/12/2015	01/05/2017	74	14/11/2020 25/04/2019
9	<b>VIMIZIM</b>	Biomarin	23/03/2015	01/12/2017	83	<b>25/11/2021</b>
10	<b>Cometriq</b>	Ipsen	24/03/2015	01/03/2016	82	
11	<b>HOLOCLAR</b>	Holostem	16/09/2015	01/08/2016	77	
12	<b>FARYDAK</b>	Novartis	22/12/2015	01/09/2016	73	

\*The date of non-funding indicated in the Nomenclátor is the first date of non-funding/exclusion.

\*\*Agreements previous November 2017 are not available.



# Orphan Drugs CIPM 2021



# Number of orphan drugs that have passed through the CIPM in 2021

202 DRUGS HAVE BEEN EVALUATED POSITIVELY <b>26 ODS(13%)</b>	71 NEW DRUGS <b>7 ODS(10%)</b>	39 NEW ACTIVE SUBSTANCES <b>6 ODS(15%)</b>
		32 OTHERS <b>1 ODS(3%)</b>
146 DRUGS HAVE BEEN EVALUATED NEGATIVELY <b>36 ODS (25%)</b>	35 NEW INDICATIONS <b>3 ODS(9%)</b>	
	52 CHANGES IN THE SUPPLY <b>6 ODS (12%)</b>	
	44 ALLEGATIONS <b>10 ODS (23%)</b>	
	70 NEW DRUGS <b>19 ODS(27%)</b>	
	26 NEW INDICATIONS <b>6 ODS(23%)</b>	
	5 ALTERNACIONES EN LA OFERTA <b>1 ODS(20%)</b>	
	45 ALLEGATIONS <b>10 ODS(22%)</b>	

# Medicines with favorable resolutions 2021

(new products and allegations)

Commercial Name	Laboratoy	NHS funding date	CIPM Meetings
New ODs			
<b>1. PREVYMIS</b>	MSD	<b>01/08/2021</b>	February 3, 2021
<b>2. ZOLGENSMA</b>	Novartis (Avexis)	<b>01/12/2021</b>	April 7, 2021
<b>3. GIVLAARI</b>	Alnylam	<b>01/09/2021</b>	May 06, 2021
<b>4. TREPULMIX</b>	SciPharm/AOP Orphan	<b>01/08/2021</b>	June 10, 2021
<b>5. EPIDYOLEX</b>	Gw Pharma	<b>01/09/2021</b>	July 08, 2021
<b>6. QUENODESOXYCHOLIC ACID</b>	Leadiant	<b>01/12/2021</b>	July 08, 2021
<b>7. KAFTRIO</b>	Vertex	<b>01/12/2021</b>	October 28, 2021
Allegations process			
<b>1. LUXTURNA</b>	Novartis	<b>01/05/2021</b>	March 03, 2021
<b>2. CABLIVI</b>	Ablynx/Sanofi	<b>01/07/2021</b>	May 06, 2021
<b>3. MEPSEVII</b>	Ultragenyx	<b>01/07/2021</b>	May 06, 2021
<b>4. POTELIGEO</b>	Kyowa Kirin	<b>01/07/2021</b>	May 06, 2021
<b>5. PREVYMIS</b>	MSD	<b>01/08/2021</b>	June 10, 2021
<b>6. GIVLAARI</b>	Alnylam	<b>01/09/2021</b>	July 08, 2021
<b>7. POLIVY</b>	Roche	<b>01/09/2021</b>	July 08, 2021
<b>8. QUENODESOXYCHOLIC ACID</b>	Leadiant	<b>01/12/2021</b>	September 30, 2021
<b>9. ZOLGENSMA</b>	Novartis (Avexis)	<b>01/12/2021</b>	October 28, 2021
<b>10. VYXEOS</b>	Jazz Pharmaceuticals	-	December 16, 2021

# Medicines with unfavorable funding resolutions (new drugs and allegations)

Commercial Name	Laboratoy	CIPM Meetings
New ODs		
1. XOSPATA	Astellas	February 03, 2021
2. POLIVY	Roche	February 03, 2021
3. LEDAGA	Recordati Rare Diseases	February 03, 2021
4. NINLARO	Takeda	February 03, 2021
5. POTELIGEO	Kyowa Kirin	March 03, 2021
6. RAXONE	Chiesi	April 7, 2021
7. VERKAZIA	Santen Pharmaceutical	April 7, 2021
8. PALYNZIQ	Biomarin	June 10, 2021
9. MYALEPTA	Amryt	June 10, 2021
10. AMGLIDIA	Ammtek	July 08, 2021
11. WAYLIVRA	Akcea	September 30, 2021
12. VYNDAQEL*	Pfizer	September 30, 2021
13. VYXEOS	Jazz	October 28, 2021
14. PEMAZYRE	Incyte	November 25, 2021
15. VIMIZIM	Biomarin	November 25, 2021
16. ISTURISA	Recordati Rare Diseases	November 25, 2021
17. TECARTUS	Gilead	December 16, 2021
18. BLENREP	GSK	December 16, 2021
19. DAURISMO	Pfizer	December 16, 2021
Allegation process		
1. LEDAGA	Recordati Rare Diseases	April 7, 2021
2. NAMUSCLA	Lupin/Exeltis	April 7, 2021
3. XOSPATA	Astellas	April 7, 2021
4. NINLARO	Takeda	April 7, 2021
5. PREVYMIS	MSD	April 7, 2021
6. LAMZEDE	Chiesi	May 06, 2021
7. RAXONE	Chiesi	July 08, 2021
8. AMGLIDIA	Ammtek	September 30, 2021
9. MYALEPTA	Amryt	November 25, 2021
10. VYNDAQEL*	Pfizer	November 25, 2021

Source: [Interministerial Commission on Drug Prices Agreements](#)

\*Vyndaqel was funded in 2013 for the treatment of transthyretin amyloidosis in adult patients with symptomatic polyneuropathy stage 1. In 2021, Vyndaqel has passed through the CIPM with a new presentation for the treatment of transthyretin amyloidosis in adult patients with cardiomyopathy.

# Unfavorable funding resolutions for new ODs 2021\*

Name	Laboratoy	NC date	Months between NC and 31/12/2021	CIPM Date (unfavorable resolutions)	Reason unfavorable condition of funding <sup>1</sup> (last CIPM agreement)
<b>1. XOSPATA</b>	Astellas	05/12/2019	25	<b>April 7, 2021</b> <b>February 3, 2021</b>	d)
<b>2. LEDAGA</b>	Helsinn (Recordati Rare Diseases)	18/04/2017	57	<b>April 7, 2021</b> <b>February 3, 2021</b>	d) y e)
<b>3. NINLARO</b>	Takeda	06/02/2017	60	<b>April 7, 2021</b> <b>February 3, 2021</b> <b>February 8, 2019</b> <b>October 31, 2018</b> <b>March 22, 2018</b>	d)
<b>4. RAXONE</b>	Chiesi	29/06/2018	43	<b>July 8, 2021</b> <b>April 7, 2021</b>	c) y d)
<b>5. VERKAZIA</b>	Santen Pharmaceuticals	08/04/2019	33	<b>April 7, 2021</b>	d) y e)
<b>6. NAMUSCLA</b>	Lupin/Exeltis	30/04/2019	33	<b>April 7, 2021</b>	d) y e)
<b>7. LAMZEDE</b> (En alegaciones)	Chiesi	07/09/2018	40	<b>May 6, 2021</b> <b>October 14, 2020</b>	d)
<b>8. MYALEPTA</b>	Amryt	06/11/2018	38	<b>June 10, 2021</b> <b>November 25, 2021</b>	c) y d)
<b>9. PALYNZIQ</b>	Biomarin	16/12/2019	25	<b>June 10, 2021</b>	c)
<b>10. AMGLIDIA</b> (En alegaciones)	Ammtek	14/01/2021	12	<b>July 8, 2021</b> <b>September 30, 2021</b>	d)
<b>11. WAYLIVRA</b>	Akcea (SOBI)	02/03/2020	22	<b>September 30, 2021</b>	c) y d)
<b>12. VYXEOS**</b>	Jazz Pharmaceuticals	19/12/2018	37	<b>October 28, 2021</b> <b>October 14, 2020</b> <b>November 28, 2019</b>	e)
<b>13. ISTURISA</b>	Recordati Rare Diseases	12/02/2021	11	<b>November 25, 2021</b>	c) y d)
<b>14. VIMIZIM</b>	BioMarin	23/03/2015	83	<b>November 25, 2021</b>	c) y d)
<b>15. PEMAZYRE</b>	Incyte	07/05/2021	8	<b>November 25, 2021</b>	c) y d)
<b>16. TECARTUS</b>	Gilead	28/12/2020	12	<b>December 16, 2021</b>	c) y d)
<b>17. BLENREP</b>	GSK	23/09/2020	15	<b>December 16, 2021</b>	c) y d)
<b>18. DAURISMO</b>	Pfizer	13/07/2020	18	<b>December 16, 2021</b>	c) y d)

## <sup>1</sup> Reasons for unfavorable condition:

- c) Therapeutic and social value of the drug and its incremental clinical benefit, taking into account its cost-effectiveness.
- d) Rationalization of public spending on pharmaceuticals and budgetary impact on the National Health System.
- e) Existence of drugs or other therapeutic alternatives for the same conditions at a lower price or lower cost. tratamiento

Source: [Interministerial Commission on Drug Prices Agreements](#)

\*ODs not funded as of 12/31/2021 that have obtained an unfavorable funding resolution during 2021.

\*\*Vyxeos obtained a favorable funding status on 12/16/2021 but as of 12/31/2021 has not yet been included in NHS funding.





**Summary  
main  
data and  
conclusions**

○ **During year 2021:**

- **20** ODs have obtained positive orphan designation in the EU.
- **19** ODs have obtained marketing authorization in the EU.
- **17** ODs have obtained National Code in Spain
- **14** ODs have been funded by the National Health System.

○ **So as of December 31, 2021 there are:**

- **176** ODs with positive orphan designation
- **129** ODs con with european marketing authorization
- **111** ODs with National Code in Spain, 86% with MA.
- **56** funded ODs, 43% with MA.

○ **EMA marketing approvals:**

- **79%** of ODs with EMA marketing authorization are authorized **under one or more special conditions.**
- Out of **21** ODs with **conditional approval** that have reached Spain only 6 (29%) are funded.

○ **Orphan drugs that have not yet reached Spain:**

- **14%** of the drugs that have MA have not yet reached Spain (18).
- **4 are advanced therapies.**
- **61%** have received MA more than one year ago.





## ○ Orphan drugs approved in 2021:

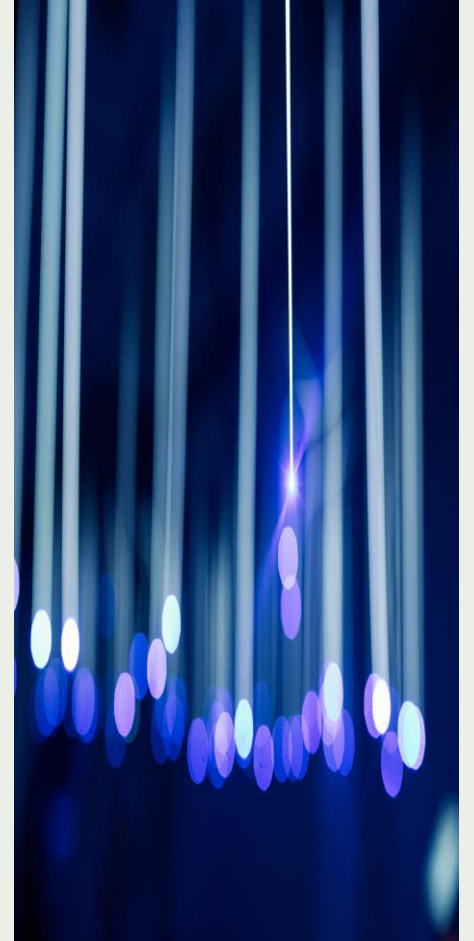
- Average waiting time to approval: **24 months**.
- **67%** have been through the CIPM 2 or more times
- **43%** have restricted funding (based on EMA-approved indications).
- **100%** have conditional funding and **43%** re subject to more than 3 conditions for funding.

## ○ Advanced Therapies with Orphan Designation:

- **12** advanced therapies with orphan designation with marketing authorization in Europe.
- **8** advanced therapies with orphan designation with NC, 67% of the MA therapies.
- **5** advanced therapies with orphan designation funded, 42% of MA therapies.
- **The average approval time** for this type of therapy is **21 months**.
- Both therapies have been approved with 3 or more confidences for funding.

## ○ Products at CIPM:

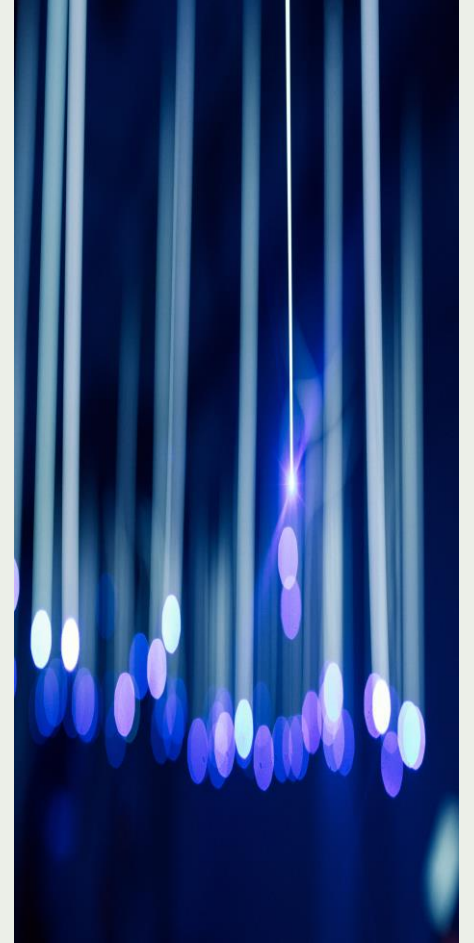
- 348 resolutions, **62** on products with orphan designation (18%).
- **10%** of the new drugs that have obtained a favorable resolution for inclusion in the NHS are orphan.
- **27%** of new drugs with unfavorable resolution are orphan.
- For **89%** of the new drugs with unfavorable resolution in 2021, the rationalization of public expenditure was the reason or one of the reasons for refusing funding.



- **UNFUNDED DRUGS:**

**55 ODs with NC are pending price and reimbursement (P/R), 2 more than in 2020, of which 51% have been awaiting a favorable financing resolution for three years or more:**

- ▶ Out of ODs with NC obtained in **2021**, 1 product has obtained a favorable financing resolution, while **94%** are still awaiting P/R. **Of these, 3 have already gone through the CIPM but have obtained an unfavorable financing resolution.**
- ▶ Likewise, of the products that obtained **NC in 2020**, **60% are currently unfunded**, a decrease of 40% with respect to 2020.
- ▶ The number of **ODs with NC in 2019** without funding has been reduced by approximately 50%, from 83% in 2020, to **42% in 2021**. Even so, **4 ODs** have obtained an **unfavorable funding resolution during 2021**.
- ▶ During **2021**, **8 ODs** that obtained **NC in 2018** have gone through the CIPM. **Of these, 50% have obtained an unfavorable funding resolution. 44% of the 2018 NC products are still pending a positive funding resolution.**
- ▶ **No changes were detected** between 2020 and 2021 in unfunded products with NC **2017** (43% unfunded), **2016** (18%) and those already outstanding between **2011** and **2015**.



- Despite the high number of new ODs coming to Spain in 2021 (70% more than in 2020), the percentage of ODs with MA and NC stands at 86%, 5 points lower than in 2019 (91%). Of the evolution of the percentages that have been analyzed over the last three years, it is the one that has dropped the most and one of those that could begin to reflect the **loss of attractiveness** of Spain for the arrival of innovation.
- The data collected in the last year reflect a slight improvement in access, approval times and financing of ODs as a result of the **efforts made by the Administration, but also due to the flexibility shown by pharmaceutical companies**. Even so, we are far from being able to say that the situation has improved, since **50% of the drugs coming in Spain do not have a favorable price and financing resolution**.
- Although all indicators show growth, it is important to remember that we were starting from a very negative situation, aggravated, no doubt, by the consequences of the Covid-19 pandemic. Against this background, the improvement in 2021 represents, in reality, a **recovery in the delays** that had accumulated in 2020, thus **requiring further progress and efforts to continue to improve by all parties involved**.
- Product **financing conditions** are becoming **increasingly stringent**. Forty-three percent are subject to more than three conditions for financing, 93% are subject to monitoring, 50% have a spending ceiling or maximum cost per patient and 21% pay-for-results/risk-sharing.
- For AELMHU, the acceleration in the research and development of new orphan drugs represents a great opportunity that should stimulate the combined efforts of all the agents involved in order to develop a **new access model** that will allow us to be at the forefront in terms of efficiency, responsiveness and attractiveness

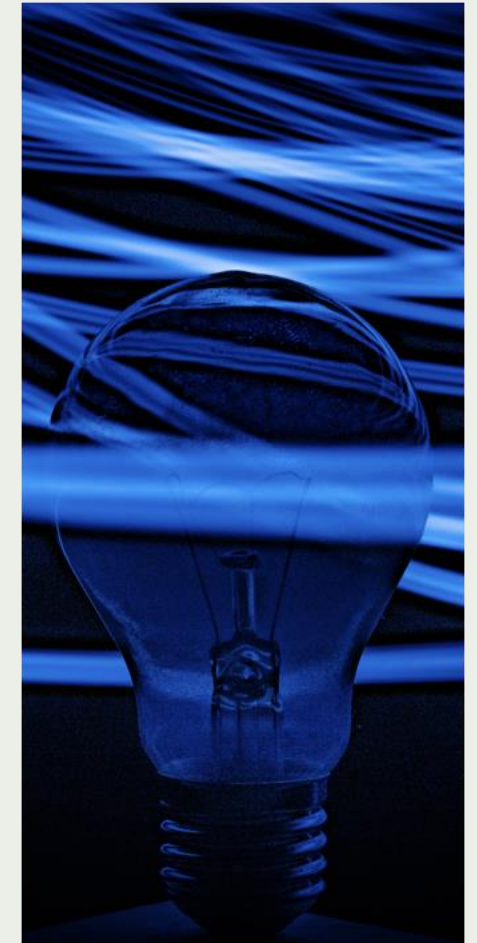


## UNFAVORABLE RESOLUTIONS

- 28% (5 ODs) of the ODs that did not receive funding during 2021, received more than one unfavorable resolution during the last year.

### Time remains a barrier to access in Spain:

- The average time that ODs have had to wait to obtain price and reimbursement in Spain (from the CN to its financing by the NHS) in 2021 is 24 months. So, the average time has decreased by 28% compared to 2020 (33 months), but it is still 72% higher than in 2019 (14 months).
- 50% of orphan drugs funded in 2021 have taken more than 2 years to get pricing and financing. Only 1 of the 14 drugs funded has taken less than 1 year.



1. List of ODs with commercial name and EMA orphan designation (176)
  - 1.1 No oncology (125)
  - 1.2 Oncology (51)
2. List of ODs with european marketing authorization (129)
  - 2.1 No oncology (90)
  - 2.2 Oncology (39)
3. List of ODs withdrawn (or not yet included in the Community Register) (47)
4. List of ODs with NC (111)
  - 4.1 No oncology (76)
  - 4.2 Oncology (35)
5. List of ODs without NC (18)
6. List of NHS funded ODs (56)
  - 6.1 No oncology (38)
  - 6.2 Oncology (18)
7. List of advanced therapies with orphan designation and European MA (12)
8. List of unfunded ODs by NHS resolution (32)
9. List of ODs under study or without funding NHS request (23)
10. List of marketed ODs not NHS funded (5)
11. Current condition of the ODs' authorization status with european marketing authorization
12. Current condition of the ODs' authorization status with NC
13. Current condition of the ODs' authorization status included in NHS funding
14. Current condition of the ODs' authorization status not included in NHS funding
15. ODs funded during 2021, situation in Europe



# I. ODs with commercial name and EMA orphan designation (1/2)

	ALSITEK
AB Science	MASICAN
	MASIPRO
ACE Pharma	ELMISOL
Ablynx/Sanofi	CABLIVI
Adienne	BEGEDINA
Advanced Accelerator Applications	LUTATHERA
	SOMAKIT
Agios	TIBSOVO
Albireo	BYLVAY
	KANUMA
Alexion	SOLIRIS
	STRENSIQ
Allos	FOLOTYN
	GIVLAARI
Alnylam	ONPATTRO
	OXLUMO
Amarin	ETHYLEICO SAPENT SOFT GELATIN CAPSULES
Amgen	BLINCYTO
	KYPROLIS

Amicus	GALAFOLD
Amryt	MYALEPTA
AMMTek	AMGLIDIA
Antigenics Therapeutics	ONCOPHAGE
Ark Therapeutics	CEREPRO
Ascendis	LONAPEG SOMATROPIN
Astellas	XOSPATA
AstraZeneca	KOSELUGO
Novartis (Avexis)	ZOLGENSMA
AVI Biopharma	EXONDYS
Basilea/Pfizer	CRESEMBA
Bausch & Lomb	RETISERT
Bayer/MSD	ADEMPAS
Bayer	NEXAVAR
Biogen	SPINRAZA
	ROCTAVIAN
	BRINEURA
BioMarin	PALYNZIQ
	VOXZOGO
	VIMIZIM
Bioprojet	WAKIX

Biovest	LYMPREVA
bluebird bio	ZYNTEGLO
	SKYSONA
Blueprin	AYVAKYT
BPL	COAGADEX
Cancer Prevention Pharma	FLYNPOVI
BMS/Celgene	IMNOVID
	ABECMA
BMS	INREBIC
	ISTODAX
	REBLOZYL
Centocor	SOVRIMA
ChemoCentryx	VYNPENTA
	LAMZEDE
Chiesi	PROCYSBI
	RAXONE
	UPKANZ
Clinuvel	SCENESSE
Clovis Oncology	XEGAFRI
CSL	IDELVION
CTI BioPharma	ENPAXIQ
Deciphera	QINLOCK
Dompe	OXERVATE
Dr Falk Pharma	JORVEZA
Endocyte Europe	FOLCEPRI

Erytech Pharma	GRASPA
Eurocept	GRANUPAS
Eurogen	ORATHECIN
EUSA Pharma	QARZIBA
	SYLVANT
FGK Representative Service	CORLUXIN
Gendux	ADVEXIN
Gentium	DEFITELIO
Genzyme	CERDELGA
	NEXVIADYME
GlaxoSmithKline	BLENREP
	ZEJULA
Global Blood Therapeutics	OXBRYTA
GW Pharma	EPIDYOLEX
Hansa Biopharma	IDEFIRIX
Helsinn (Recordati Rare Diseases)	LEDAGA
Holostem	HOLOCLAR
HRA Pharma Rare Diseases	KETOCONAZOLE
Immedica/SOBI	RAVICTI
	PEMAZYRE
Incyte	MINJUVI
	ICLUSIG
Insmed	ARIKAYCE
Intercept	OCALIVA



# I. ODs with commercial name and EMA orphan designation (2/2)

Ipsen	COMETRIQ	Mylan	PRETOMANID	Phama Mar	APLIDIN	SOBI	ALPROLIX
	XERMELO		TOBI	Pharmaxis (Chiesi)	BRONCHITOL		ASPAVELI
Janssen	DACOGEN	MYR (Gilead)	HEPCLUDEX	Pharm Research Associates	SURFAXIN	Akcea (SOBI)	TEGSEDI
	DARZALEX	Neurochem	KIACTA				WAYLIVRA
	OPSUMIT	Nordic	SPANIDIN			Soligenix	ORBEC
	SIRTURO	Nova Laboratories	XALUPRINE			Promethera	HEPARESC
Jazz Pharma	VYXEOS	Novo Nordisk	SOGROYA	Protherics	VORAXAZE	Shire (Takeda)	REVESTIVE
Kamada	INFINIA	Novartis	ADAKVEO	PTC Therapeutics	TRANSLARNA	Takeda	ADCETRIS
Kite Pharma	TECARTUS		KYMRIAH	Recordati Rare Diseases	CYSTADROPS		ALOFISEL
	YESCARTA		LUXTURNA		ISTURISA		NATPAR
Kyowa Kirin	CRYSVITA		RYDAPT	Rhythm Pharmaceuticals	SIGNIFOR		NINLARO
	POTELIGEO		VOTUBIA		IMCIVREE		TAKHZYRO
Laboratoires CTRS	ORPHACOL		Oncoscience	THERALOC	Roche		ENSPRYNG
Leadiant	ÁCIDO QUENODES-OXICÓLICO	Orbona	LOULLA	EVRYSDI		Ultragenyx	MEPSEVII
		Orchard Therapeutics	LIBMELDY	GAZYVARO	Unither Therapeutik	TYVASO	
			STRIMVELIS	POLIVY	Vanda Pharmaceuticals	HETLIOZ	
Lucane	DROPCYS	Otsuka	DELTYBA	Santey	VERKAZIA	Vertex	KAFTRIO
	PROHIPUR	Pfizer	BESPONSA	SciPharm/AOP Orphan	TREPULMIX		KALYDECO
Lupin/Exeltis	NAMUSCLA		DAURISMO	Novartis	FARYDAK	SYMKEVI	
Medac	TRECONDI		ELELYSO	Servier	ONIVYDE	Viela Bio	UPLIZNA
MediWound	NEXOBRID		MYLOTARG	SFL Pharmaceuticals	OBILTOXAXIMAB	Viridian	BLECTIFOR
MolMed	ZAFIRIDE		NGENLA			Zogenix/MSD	FINTEPLA
MSD	PREVYMIS		VYNDAQEL				

# 1.1 ODs with commercial name and EMA orphan designation (no oncology)

AB Science	ALSITEK
	MASIPRO
Ablynx/Sanofi	CABLIVI
ACE Pharmaceuticals	ELMISOL
Adienne	BEGEDINA
Albeiro	BLYVAY
	KANUMA
Alexion	SOLIRIS
	STRENSIQ
Alnylam	GIVLAARI
	ONPATTRO
	OXLUMO
Amarin	ETHYLEICO SAPENT SOFT GELATIN CAPSULES
Amicus	GALAFOLD
Amryt	MYALEPTA
AMMTeK	AMGLIDIA
Ascendis	LONAPEGSOMATROPIN
Novartis (Avexis)	ZOLGENSMA
AVI Biopharma	EXONDYS

Basilea/Pfizer	CRESEMBA
Bausch & Lomb	RETISERT
Bayer/MSD	ADEMPAS
Biogen	SPINRAZA
	BRINEURA
	PALYNZIQ
BioMarin	ROCTAVIAN
	VOXZOGO
	VIMIZIM
Bioprojet	WAKIX
bluebird bio	SKYSONA
	ZYNTEGLO
BPL	COAGADEX
BMS	INREBIC
	REBLOZYL
Centocor	SOVRIMA
ChemoCentryx	VYNPENTA
	LAMZEDE
	PROCYSBI
Chiesi	RAXONE
	UPKANZ
	SCENESSE

Clovis Oncology	XEGAFRI
CSL	IDELVION
CTI BioPharma	ENPAXIQ
Dompe	OXERVATE
Dr Falk Pharma	JORVEZA
Eurocept	GRANUPAS
EUSA Pharma	SYLVANT
FGK Representative Service	CORLUXIN
Gendux	ADVEXIN
Gentium	DEFITELIO
Genzyme	CERDELGA
	NEXVIADYME
Global Blood Therapeutics	OXBRYTA
GW Pharma	EPIDYOLEX
Hansa Biopharma	IDEFIRIX
Holostem	HOLOCLAR
HRA Pharma Rare Diseases	KETOCONAZOLE
Immedica/SOBI	RAVICTI
Insmed	ARIKAYCE
Intercept	OCALIVA
Ipsen	XERMELO



# 1.1 ODs with commercial name and EMA orphan designation (no oncology)

Janssen	OPSUMIT
	SIRTURO
Kamada	INFINIA
Kyowa Kirin	CRYSVITA
Laboratoires CTRS	ORPHACOL
Leadiant	ÁCIDO QUENODES- OXICÓLICO
Lucane	DROPCYS
	PROHIPUR
Lupin/Exeltis	NAMUSCLA
Medac	TRECONDI
MediWound	NEXOBRID
MSD	PREVYMIS
Mylan	PRETOMANID
	TOBI
MYR (Gilead)	HEPCLUDEX
Neurochem	KIACTA
Nordic	SPANIDIN
	ADAKVEO
Novartis	LUXTURNA
	VOTUBIA

Orchard Therapeutics	LIBMELDY
	STRIMVELIS
Otsuka	DELYBA
	ELELYSO
Pfizer	NGENLA
	VYNDAQEL
Pharmaxis (Chiesi)	BRONCHITOL
Pharm Research Associates	SURFAXIN
Promethera	HEPARESC
Protherics	VORAXAZE
PTC Therapeutics	TRANSLARNA
	CYSTADROPS
Recordati Rare Diseases	ISTURISA
	SIGNIFOR
Rhythm Pharmaceuticals	IMCIVREE
	ENSPRYNG
Roche	EVRYSDI
	ESBRIET
Santey	VERKAZIA
SciPharm/AOP Orphan	TREPULMIX
SFL Pharmaceuticals	OBILTOXAXIMAB

SOBI	ALPROLIX
	ASPAVELI
Akcea (SOBI)	TEGSEDI
	WAYLIVRA
Soligenix	ORBEC
Shire (Takeda)	REVESTIVE
	ALOFISEL
	NATPAR
Takeda	TAKHZYRO
	VPRIV
Toray	WINFURAN
Ultragenyx	MEPSEVII
Unither Therapeutik	TYVASO
Vanda Pharmaceuticals	HETLIOZ
	KAFTRIO
Vertex	KALYDECO
	SYMKEVI
Viela Bio	UPLIZNA
Viridian	BLECTIFOR
Zogenix/MSD	FINTEPLA

# 1.2 ODs with commercial name and EMA orphan designation (oncology)

AB Science	MASICAN
Advanced Accelerator Applications	LUTATHERA
	SOMAKIT
Agios	TIBSOVO
Allos	FOLOTYN
	BLINCYTO
Amgen	KYPROLIS
Antigenics Therapeutics	ONCOPHAGE
Ark Therapeutics	CEREPRO
Astellas	XOSPATA
AstraZeneca	KOSELUGO
Bayer	NEXAVAR
Biovest	LYMPREVA
Blueprin	AYVAKYT
Cancer Prevention Pharma	FLYNPOVI
BMS/Celgene	IMNOVID
	ABECMA
Celgene	ISTODAX
Deciphera	QINLOCK

Endocyte Europe	FOLCEPRI
Erytech Pharma	GRASPA
Eurogen	ORATHECIN
EUSA Pharma	QARZIBA
	BLENREP
GlaxoSmithKline	ZEJULA
Helsinn (Recordati Rare Diseases)	LEDAGA
	PEMAZYRE
Incyte	MINJUVI
	ICLUSIG
Ipsen	COMETRIQ
	DACOGEN
Janssen	DARZALEX
Jazz Pharmaceuticals	VYXEOS
	TECARTUS
Kite Pharma	YESCARTA
Kyowa Kirin	POTELIGEO

MolMed	ZAFIRIDE
Nova Laboratories	XALUPRINE
	KYMRIAH
Novartis	RYDAPT
Oncoscience	THERALOC
Orbona	LOULLA
	BESPONSA
Pfizer	DAURISMO
	MYLOTARG
Phama Mar	APLIDIN
	GAZYVARO
Roche	POLIVY
Novartis	FARYDAK
Servier	ONIVYDE
Stemline	ELZONRIS
	ADCETRIS
Takeda	NINLARO

## 2. ODs with european marketing authorization (1/2)

Ablynx/Sanofi	CABLIVI
Advanced Accelerator Applications	LUTATHERA
	SOMAKIT
Albeiro	BLYVAY
Alexion	KANUMA
	SOLIRIS
	STRENSIQ
Alnylam	GIVLAARI
	ONPATTRO
	OXLUMO
Amgen	BLINCYTO
	KYPROLIS
Amivas	ARTESUNATE

Amicus	GALAFOLD
Amryt	MYALEPTA
AMMTeK	AMGLIDIA
Astellas	XOSPATA
AstraZeneca	KOSELUGO
Novartis (Avexis)	ZOLGENSMA
Basilea/Pfizer	CRESEMBA
Bayer/MSD	ADEMPAS
Bayer	NEXAVAR
Biogen	SPINRAZA
BioMarin	BRINEURA
	PALYNZIQ
	VIMIZIM
	VOXZOGO
Bioprojet	WAKIX
bluebird bio	SKYSONA
	ZYNTEGLO
Blueprin	AYVAKYT

BPL	COAGADEX
Deciphera	QINLOCK
BMS/Celgene	IMNOVID
BMS	ABECMA
	INREBIC
	REBLOZYL
Chiesi	LAMZEDE
	RAXONE
	PROCYSBI
Clinuvel	SCENESSE
CSL	IDELVION
Dompe	OXERVATE
Dr Falk Pharma	JORVEZA
Eurocept	GRANUPAS
EUSA Pharma	QARZIBA
	SYLVANT
Gentium	DEFITELIO
Genzyme	CERDELGA

GlaxoSmithKline	BLENREP
	ZEJULA
GW Pharma	EPIDYOLEX
Hansa Biopharma	IDEFIRIX
Helsinn (Recordati Rare Diseases)	LEDAGA
Holostem	HOLOCLAR
HRA Pharma Rare Diseases	KETOCONAZOLE
Immedica/SOBI	RAVICTI
	PEMAZYRE
Incyte	MINJUVI
	ICLUSIG
Insmed	ARIKAYCE
Intercept	OCALIVA
Ipsen	COMETRIQ
	XERMELO

## 2. ODs with european marketing authorization (2/2)

Janssen	DACOGEN
	DARZALEX
	OPSUMIT
	SIRTURO
Jazz Pharmaceuticals	VYXEOS
Kite Pharma	TECARTUS
	YESCARTA
Kyowa Kirin	CRYSVITA
	POTELIGEO
Laboratoires CTRS	ORPHACOL
Leadiant	ÁCIDO QUENODES- OXICÓLICO
Lupin/Exeltis	NAMUSCLA
Medac	TRECONDI

MediWound	NEXOBRID
MSD	PREVYMIS
Mylan	PRETOMANID
	TOBI
MYR (Gilead)	HEPCLUDEX
Nova Laboratories	XALUPRINE
Novartis	KYMRIAH
	ADAKVEO
	LUXTURNA
	RYDAPT
	VOTUBIA
Novo Nordisk	SOGROYA
Orchard Therapeutics	LIBMELDY
	STRIMVELIS
Otsuka	DELTYBA
Pharmaxis (Chiesi)	BRONCHITOL
Pfizer	BESPONSA
	DAURISMO
	MYLOTARG
	VYNDAQEL

PTC Therapeutics	TRANSLARNA
	CYSTADROPS
Recordati Rare Diseases	ISTURISA
	SIGNIFOR
Rhythm Pharmaceuticals	IMCIVREE
Roche	EVRYSDI
	ENSPRYNG
	GAZYVARO
	POLIVY
Santey	VERKAZIA
SciPharm/AOP Orphan	TREPULMIX
Novartis	FARYDAK
Servier	ONIVYDE
SFL Pharmaceuticals	OBILTOXAXIMAB
	ALPROLIX
SOBI	ASPAVELI
	TEGSEDI
AKCEA (SOBI)	WAYLIVRA
STEMLINE	ELZONRIS

Shire (Takeda)	REVESTIVE
Takeda	ADCETRIS
	ALOFISEL
	NATPAR
	NINLARO
	TAKHZYRO
	VPRIV
Ultragenyx	MEPSEVII
Vanda Pharmaceuticals	HETLIOZ
	KAFTRIO
Vertex	KALYDECO
	SYMKEVI
Zogenix/MSD	FINTEPLA

## 2.1 ODs with european marketing authorization (no oncology)

Ablynx/Sanofi	CABLIVI
Albireo	BYLVAY
	KANUMA
Alexion	SOLIRIS
	STRENSIQ
	GIVLAARI
Alnylam	ONPATTRO
	OXLUMO
Amicus	GALAFOLD
Amivas	ARTESUNAT E
Amryt	MYALEPTA
AMMTeK	AMGLIDIA
Novartis (Avexis)	ZOLGENSM A
Basilea/Pfizer	CRESEMBA
Bayer/MSD	ADEMPAS
Biogen	SPINRAZA
	BRINEURA
	PALYNZIQ
BioMarin	VOXZOGO
	VIMIZIM
Bioprojet	WAKIX
	SKYSONA
bluebird bio	ZYNTEGLO

BPL	COAGADEX
	INREBIC
BMS	REBLOZYL
	LAMZEDE
Chiesi	PROCYSBI
	RAXONE
Clinuvel	SCENESSE
CSL	IDELVION
Dompe	OXERVATE
Dr Falk Pharma	JORVEZA
Eurocept	GRANUPAS
EUSA Pharma	SYLVANT
Gentium	DEFITELIO
Genzyme	CERDELGA
GW Pharma	EPIDYOLEX
Hansa Biopharma	IDEFIRIX
Holostem	HOLOCLAR
HRA Pharma Rare Diseases	KETOCONAZO LE
Immedica/SOBI	RAVICTI
Insmed	ARIKAYCE
Intercept	OICALIVA
Ipsen	XERMELO

	OPSUMIT
Janssen	SIRTURO
Kyowa Kirin	CRYSVITA
Laboratoires CTRS	ORPHACOL
	ÁCIDO QUENODES- OXICÓLICO
Leadiant	
Lupin/Exeltis	NAMUSCLA
Medac	TRECONDI
MediWound	NEXOBRID
MSD	PREVYMIS
	PRETOMANID
Mylan	TOBI
MYR (Gilead)	HEPCLUDEX
	ADAKVEO
Novartis	LUXTURNA
	VOTUBIA
Novo Nordisk	SOGROYA
Orchard Therapeutics	LIBMELDY
	STRIMVELIS
Otsuka	DELTYBA
Pharmaxis (Chiesi)	BRONCHITOL
Pfizer	VYNDAQEL
PTC Therapeutics	TRANSLARNA

	CYSTADROPS
Recordati Rare Diseases	ISTURISA
	SIGNIFOR
Rhythm Pharmaceuticals	IMCIVREE
	ENSPRYNG
Roche	EVYRSDI
Santen	VERKAZIA
SciPharm/AOP Orphan	TREPULMIX
SFL Pharmaceuticals	OBILTOXAXIM AB
	ALPROLIX
SOBI	ASPAVELI
	TEGSEDI
AKCEA (SOBI)	WAYLIVRA
Shire (Takeda)	REVESTIVE
	ALOFISEL
	NATPAR
Takeda	TAKHZYRO
	VPRIV
Ultragenyx	MEPSEVII
Vanda Pharmaceuticals	HETLIOZ
	KAFTRIO
Vertex	KALYDECO
	SYMKEVI
Zogenix/MSD	FINTEPLA

## 2.2 ODs with european marketing authorization (oncology)

Advanced Accelerator Applications	LUTATHERA SOMAKIT
Amgen	BLINCYTO KYPROLIS
Astellas	XOSPATA
AstraZeneca	KOSELUGO
Bayer	NEXAVAR
Blueprin	AYVAKYT
Deciphera	QINLOCK
BMS/Celgene	IMNOVID
Celgene	ABECMA
EUSA Pharma	QARZIBA
GlaxoSmithKline	BLENREP ZEJULA
Helsinn (Recordati Rare Diseases)	LEDAGA
Incyte	ICLUSIG MINJUVI PEMAZYRE
Ipsen	COMETRIQ
Janssen	DACOGEN DARZALEX

Jazz Pharmaceuticals	VYXEOS
	TECARTUS
Kite Pharma	YESCARTA
Kyowa Kirin	POTELIGEO
Nova Laboratories	XALUPRINE
Novartis	KYMRIAH RYDAPT
	BESPONSA
Pfizer	DAURISMO MYLOTARG
Pharma Mar	APLIDIN
Roche	GAZYVARO POLIVY
Novartis	FARYDAK
Servier	ONIVYDE
Stemline	ELZONRIS
Takeda	ADCETRIS NINLARO

### 3. ODs withdrawn (or not yet included in the Community Register)

	ALSITEK (2)	Cancer Prevention Pharma	FLYNPOVI (7)	Neurochem	KIACTA (1)
AB Science	MASICAN (2)		ISTODAX (2)	Nordic	SPANIDIN (1)
	MASIPRO (2)	Centocor	SOVRIMA (2)	Oncoscience	THERALOC (1)
ACE Pharmaceuticals	ELMISOL (1)	ChemoCentryx	VYNPENTA (1)	Orbona	LOULLA (1)
Adienne	BEGEDINA (1)	Chiesi	UPKANZ (1)	Pfizer	NGENLA (4)
Agios	TIBSOVO (1)	Clovis Oncology	XEGAFRI (1)		ELELYSO (2)
Allos	FOLOTYN (2)	CTI BioPharma	ENPAXIQ (1)	Phama Mar	APLIDIN (2)
		Endocyte Europe	FOLCEPRI (1)	Pharm Research Associates	SURFAXIN (1)
Amarin	ETHYLEICO SAPENT SOFT GELATIN CAPSULES (1)	Erytech Pharma	GRASPA (1)	Promethera	HEPARESC (2)
		Eurogen	ORATHECIN (1)	Protherics	VORAXAZE (1)
Antigenics Therapeutics	ONCOPHAGE (1)	FGK Representative Service	CORLUXIN (1)	Recordati Rare Diseases	CARBAGLU (6)
Ark Therapeutics	CEREPRO (1)	Gendux	ADVEXIN (1)	Roche	ESBRIET (6)
Ascendis	LONAPEG SOMATROPIN(4)	Genzyme	MOZOBIL (6)	Shire	PLENADREN (6)
AstraZeneca	LUMOXITI (1)	Global Blood Therapeutics	OXBRYTA (4)	Soligenix	ORBEC (1)
AVI Biopharma	EXONDYS (2)	Janssen	IMBRUVICA (6)	Stemline	ELZONRIS (3)
Bausch & Lomb	RETISERT (1)	Kamada	INFINIA (1)	Toray	WINFURAN (1)
BeiGene	BRUKINSA (6)		DROPCYS (2)	uniQure	GLYBERA (5)
BioMarin	ROCTAVIAN (1)	Lucane	PROHIPUR (1)	Unither Therapeutik	TYVASO (1)
Biovest	LYMPREVA (2)	MolMed	ZAFIRIDE (1)	Viela Bio	UPLIZNA
				Viridian	BLECTIFOR (1)

(1) Withdrawn of the MA by laboratory choice

(2) Refused MA

(3) No trade name in the Community Register of Orphan Medicinal Products

(4) No MA yet (as of 31. 12. 21)

(5) Withdrawn from use in the European Union

(6) OD Expired

(7) CHMP negative

## 4. ODs with NC

Abylnx/Sanofi	CABLIVI
Advanced Accelerator Applications	LUTATHERA
	SOMAKIT
Albireo	BYLVAY
	KANUMA
Alexion	SOLIRIS
	STRENSIQ
Alnylam	GIVLAARI
	ONPATTRO
	OXLUMO
Amgen	KYPROLIS
	BLINCYTO
Amicus	GALAFOLD
Amryt	MYALEPTA
Ammtek	AMGLIDIA
Astellas	XOSPATA
AstraZeneca	KOSELUGO
Novartis (Avexis)	ZOLGENSMA
Basilea/Pfizer	CRESEMBA
Bayer/MSD	ADEMPAS
Bayer	NEXAVAR
Biogen	SPINRAZA
	BRINEURA
	PALYNZIQ
Biomarin	VIMIZIM

Bioprojet	WAKIX
BMS/Celgene	IMNOVID
BMS	INREBIC
	REBLOZYL
Chiesi	LAMZEDE
	PROCYSBI
	RAXONE
Clinuvel	SCENESSE
CSL	IDELVION
Dompe	OXERVATE
Dr. Falk Pharma	JORVEZA
Eurocept	GRANUPAS
Eusa Pharma	QARZIBA
	SYLVANT
Genzyme	CERDELGA
Glaxosmithkline	BLENREP
	ZEJULA
Gw Pharma	EPIDYOLEX
Hansa	IDEFIRIX
Helsinn (Recordati Rare Diseases)	LEDAGA
Holostem	HOLOCLAR
Immedica/SOBI	RAVICTI
Incyte	ICLUSIG
	MINJUVI
	PEMAZYRE
Intercept	OCALIVA
Ipsen	Cometriq
	XERMELO

Janssen	DACOGEN
	DARZALEX
	OPSUMIT
Jazz	SIRTURO
	VYXEOS
Kite Pharma	TECARTUS
	YESCARTA
Kyowa Kirin	POTELIGEO
	CRYSVITA
Lab. Ctrs	ORPHACOL
Leadiant	ÁCIDO QUENODES-OXICÓLICO
	NAMUSCLA
Medac GMBH	TRECONDI
Mediwound	NEXOBRID
MSD	PREVYMIS
Mylan	TOBI
MYR (Gilead)	HEPCLUDEX
Nova Laboratories	XALUPRINE
	FARYDAK
	ADAKVEO
Novartis	KYMRIAH
	RYDAPT
	LUXTURNA
	VOTUBIA
Orchard Therapeutics	LIBMELDY
Otsuka	DELTYBA
Pharmaxis (Chiesi)	BRONCHITOL
	BESPONSA
Pfizer	DAURISMO
	MYLOTARG
	VYNDAQEL

Ptc Therapeutics	TRANSLARNA
Recordati Rare Diseases	ISTURISA
	CYSTADROPS
Roche	SIGNIFOR
	GAZVVARO
	POLIVY
Santen	ENSPRYNG
	EVRYSDI
Santen	VERKAZIA
SciPharm/AOP Orphan	TREPULMIX
Servier	ONIVYDE
SOBI	ALPROLIX
	ASPAVELI
AKCEA (SOBI)	TEGSEDI
	WAYLIVRA
Shire (Takeda)	REVESTIVE
	ADCETRIS
Takeda	NINLARO
	ALOFISEL
	NATPAR
	TAKHZYRO
Ultragenyx	VPRIV
	MEPSEVII
Vertex	KAFTRIO
	KALYDECO
Zogenix/MSD	SYMKEVI
	FINTEPLA



## 4.1 ODs with NC (no oncology)

Ablynx/Sanofi	CABLIVI
Albireo	BYLVAY
	KANUMA
Alexion	SOLIRIS
	STRENSIQ
	GIVLAARI
Alnylam	ONPATTRO
	OXLUMO
Amicus	GALAFOLD
Amryt	MYALEPTA
Ammtek	AMGLIDIA
Novartis (Avexis)	ZOLGENSMA
Basilea/Pfizer	CRESEMBA
Bayer/MSD	ADEMPAS
Biogen	SPINRAZA

	BRINEURA
Biomarin	PALYNZIQ
	VIMIZIM
Bioprojet	WAKIX
BMS	REBLOZYL
	INREBIC
	LAMZEDE
Chiesi	RAXONE
	PROCYSBI
Clinuvel	SCENESSE
CSL	IDELVION
Dompe	OXERVATE
Dr. Falk Pharma	JORVEZA
Eurocept	GRANUPAS
Eusa Pharma	SYLVANT
Genzyme	CERDELGA
Gw Pharma	EPIDYOLEX
Hansa	IDEFIRIX
Holostem	HOLOCLAR

Immedica/SOBI	RAVICTI
Intercept	OCALIVA
Ipsen	XERMELO
	OPSUMIT
Janssen	SIRTURO
Kyowa Kirin	CRYSVITA
Laboratoires Ctrs	ORPHACOL
Leadiant	QUENODESOXYCHOLIC ACID
Lupin/Exeltis	NAMUSCLA
Mediwound	NEXOBRID
MSD	PREVYMIS
Mylan	TOBI
MYR (Gilead)	HEPCLUDEX
	ADAKVEO
Novartis	LUXTURNA
	VOTUBIA
Orchard Therapeutics	LIBMELDY
Otsuka	DELTYBA
Pharmaxis (Chiesi)	BRONCHITOL
Pfizer	VYNDAQEL
Ptc Therapeutics	TRANSLARNA

	CYSTADROPS
Recordati Rare Diseases	ISTURISA
	SIGNIFOR
Roche	ENSPRYNG
	EVRYSDI
Santen	VERKAZIA
SciPharm/AOP Orphan	TREPULMIX
	ALPROLIX
SOBI	ASPAVELI
	TEGSEDI
AKCEA (SOBI)	WAYLIVRA
Shire (Takeda)	REVESTIVE
	ALOFISEL
	NATPAR
Takeda	TAKHZYRO
	VPRIV
Ultragenyx	MEPSEVII
	KAFTRIO
Vertex	KALYDECO
	SYMKEVI
Zogenix/MSD	FINTEPLA

## 4.2 ODs with NC (oncology)

Advanced Accelerator Applications	LUTATHERA SOMAKIT
Amgen	BLINCYTO KYPROLIS
Astellas	XOSPATA
AstraZeneca	KOSELUGO
Bayer	NEXAVAR
BMS/Celgene	IMNOVID
Eusa Pharma	QARZIBA
Glaxosmithkline	BLNREP ZEJULA
Helsinn (Recordati Rare Diseases)	LEDAGA
Incyte	ICLUSIG MINJUVI PEMAZYRE
Ipsen	Cometriq
Janssen	DACOGEN DARZALEX

Jazz Pharmaceuticals	VYXEOS
Kite Pharma	TECARTUS YESCARTA
Kyowa Kirin	POTELIGEO
Medac GMBH	TRECONDI
Nova Laboratories	XALUPRINE FARYDAK
Novartis	KYMRIAH RYDAPT
Pfizer	BESPONSA DAURISMO MYLOTARG
Roche	GAZYVARO POLIVY
Servier	ONIVYDE
Takeda	ADCETRIS

## 5. ODs without NC

Amivas	ARTESUNATE
Blued bio	SKYSONA ZYNTEGLO
Blueprint	AYVAKYT
BioMarin	VOXZOGO
BPL	COAGADEX
Deciphera	QINLOCK
Celgene	ABECMA
Gentium	DEFITELIO
HRA Pharma	KETOCONAZOLE
Insmed	ARIKAYCE
Mylan	PRETOMANID
Novo Nordisk	SOGROYA
Orchard Therapeutics	STRIMVELIS
Rhythm Pharmaceuticals	IMCIVREE
SFL Pharmaceuticals	OBILTOXAXIMAB
Stemlinebir	ELZONRIS
Vanda Pharmaceuticals	HETLIOZ

## 6. ODs NHS funded

Ablynx/Sanofi	CABLIVI
Advanced Accelerator Applications	LUTATHERA
	SOMAKIT
Alexion	KANUMA
	SOLIRIS
Alnylam	GIVLAARI
	ONPATTRO
Amgen	KYPROLIS
Amicus	GALAFOLD
SciPharm/AOP Orphan	TREPULMIX
Novartis (Avexis)	ZOLGENSMA
Basilea/Pfizer	CRESEMBA
Bayer/MSD	ADEMPAS
Bayer	NEXAVAR
Biogen	SPINRAZA
Bioprojet	WAKIX
BMS/Celgene	IMNOVID
CSL	IDELVION

Eusa Pharma	SYLVANT
Genzyme	CERDELGA
Glaxosmithkline	ZEJULA
Gw Pharma	EPIDYOLEX
Immedica/SOBI	RAVICTI
Incyte	ICLUSIG
Intercept	OCALIVA
	DACOGEN
Janssen	DARZALEX
	OPSUMIT
Kite Pharma	YESCARTA
Kyowa Kirin	CRYSVITA
	POTELIGEO
Laboratoires Ctrs	ORPHACOL
Leadiant	QUENODESOXYCHOLIC ACID
MSD	PREVYMIS
	RYDAPT
Novartis	LUXTURNA
	KYMRIAH
	VOTUBIA

Otsuka	DELTYBA
	BESPONSA
Pfizer	MYLOTARG
	VYNDAQEL
Recordati Rare Diseases	SIGNIFOR
Roche	GAZYVARO
	POLIVY
Shire	TAKHZYRO
SOBI	ALPROLIX
AKCEA (SOBI)	TEGSEDI
Shire (Takeda)	REVESTIVE
	ADCETRIS
Takeda	ALOFISEL
	VPRIV
Ultragenyx	MEPSEVII
Vertex	KALYDECO
	SYMKEVI
	KAFTRIO

## 6.1 ODs NHS funded (no oncology)

Ablynx/Sanofi	CABLIVI
Alexion	KANUMA
	SOLIRIS
Alnylam	GIVLAARI
	ONPATTRO
Amicus	GALAFOLD
SciPharm/AOP Orphan	TREPULMIX
Novartis (Avexis)	ZOLGENSMA
Basilea/Pfizer	CRESEMBA
Bayer/MSD	ADEMPAS
Biogen	SPINRAZA
Bioprojet	WAKIX
CSL	IDELVION
Eusa Pharma	SYLVANT
Genzyme	CERDELGA
Gw Pharma	EPIDYOLEX
Immedica/SOBI	RAVICTI

Intercept	OCALIVA
Janssen	OPSUMIT
Kyowa Kirin	CRYSVITA
Laboratoires Ctrs	ORPHACOL
Leadiant	QUENODESOXYCHOLIC ACID
MSD	PREVMIS
Novartis	LUXTURNA
	VOTUBIA
Otsuka	DELTYBA
Pfizer	VYNDAQEL
Recordati Rare Diseases	SIGNIFOR
Shire	TAKHZYRO
SOBI	ALPROLIX
AKCEA (SOBI)	TEGSEDI
Shire (Takeda)	REVESTIVE
	ALOFISEL
Takeda	VPRIV
	MEPSEVII
Ultragenyx	KALYDECO
	KAFTRIO
	SYMKEVI

## 6.2 ODs NHS funded (oncology)

Advanced Accelerator Applications	LUTATHERA SOMAKIT
Amgen	KYPROLIS
Bayer	NEXAVAR
BMS/Celgene	IMNOVID
Glaxosmithkline	ZEJULA
Incyte	ICLUSIG
Janssen	DACOGEN DARZALEX
Kite Pharma	YESCARTA
Kyowa Kirin	POTELIGEO
Novartis	KYMRIAH RYDAPT
Pfizer	BESPONSA MYLOTARG
Roche	GAZYVARO POLIVY
Takeda	ADCETRIS

## 7. Advanced therapies with orphan designation and MA

LABORATORY	COMMERCIAL NAME	NHS FUNDING
Bluebird bio	SKYSONA	Not funded
Bluebird bio	ZYNTEGLO	Not funded
Bristol-myers squibb pharma	ABECMA	Not funded
Holostem terapie avanzate	HOLOCLAR	Not funded
Kite pharma	YESCARTA	Funded
Kite pharma	TECARTUS	Not funded
Novartis	KYMRIAH	Funded
Novartis	LUXTURNA	Funded
Novartis	ZOLGENSMA	Funded
Orchard therapeutics	STRIMVELIS	Not funded
Orchard therapeutics	LIBMELDY	Not funded
Takeda pharma	ALOFISEL	Funded

## 8. Unfunded ODs by NHS resolution

		Time non-financing*
Alexion	STRENSIQ	76
Amgen	BLINCYTO	74
Ammtek	AMGLIDIA	12
Amryt	MYALEPTA	38
Astellas	XOSPATA	25
Biomarin	PALYNZIQ	25
	VIMIZIM	83
Chiesi	LAMZEDE	40
	RAXONE	43
	PROCYSBI	44
Clinuvel	SCENESSE	58
Dompe	OXERVATE	52

		Time non-financing*
Dr. Falk Pharma	JORVEZA	47
Eurocept	GRANUPAS	92
Helsinn (Recordati Rare Diseases)	LEDAGA	57
Holostem	HOLOCLAR	77
Ipsen	COMETRIQ	82
	XERMELO	49
Janssen	SIRTURO	89
Jazz Pharmaceuticals	VYXEOS	37
Lupin/Exeltis	NAMUSCLA	33
Mediwound	NEXOBRID	89

		Time non-financing*
Mylan	TOBI	126
Nova Laboratories	XALUPRINE	108
Novartis	FARYDAK	73
Pharmaxis (Chiesi)	BRONCHITOL	67
Ptc Therapeutics	TRANSLARNA	86
Recordati Rare Diseases	CYSTADROPS	49
Santen	VERKAZIA	33
Servier	ONIVYDE	63
Takeda	NATPAR	47
	NINLARO	60



## 9. ODs under study or without funding: NHS request

		Time non-financing*
Albeiro	BYLVAY	0
Alnylam	OXLUMO	12
AstraZeneca	KOSELUGO	5
Biomarin	BRINEURA	31
	PALYNZIQ	25
BMS	INREBIC	10
	REBLOZYL	17

		Time non-financing*
Eusa Pharma	QARZIBA	40
Glaxosmithkline	BLENREP	15
	PEMAZYRE	8
Incyte	MINJUVI	3
	TECARTUS	12
Medac GMBH	TRECONDI	11

		Time non-financing*
Novartis	ADAKVEO	11
Orchard Therapeutics	LIBMELDY	2
Pfizer	DAURISMO	18
Recordati Rare Diseases	ISTURISA	11
Roche	ENSPRYNG	5
	EVRYSDI	8
SOBI	WAYLIVRA	22
	ASPAVELI	0
Zogenix/MSD	FINTEPLA	7

## 10. Marketed ODs not NHS funded

Pharmaxis (Chiesi)	BRONCHITOL
Chiesi	PROCYSBI
Ipsen	XERMELO
Mediwound	NEXOBRID
Mylan	TOBI

All these drugs are non-oncological.

## II. Current condition of the ODs' authorization status with European marketing authorization

ACCELERATED EVALUATION	
Albireo	BYLVAY
Alexion	KANUMA SOLIRIS
Alnylam	ONPATTRO
Amgen	KYPROLIS
Biogen	SPINRAZA
BioMarin	BRINEURA
bluebird bio	ZYNTEGLO
BPL	COAGADEX
Dompe	OXERVATE
Dr Falk Pharma	JORVEZA
EUSA Pharma	SYLVANT
HRA Pharma Rare Diseases	KETOCONAZOLE
Incyte	ICLUSIG
Janssen	DARZALEX
Roche	EVRYSDI
Santen	VERKAZIA
AKCEA (SOBI)	TEGSEDI
Takeda	TAKHZYRO VPRIV
Vertex	KALYDECO

CONDITIONAL AUTHORIZATION	
Novartis (Avexis)	ZOLGENSMA
AstraZeneca	KOSELUGO
bluebird bio	ZYNTEGLO
Celgene	ABECMA
GlaxoSmithKline	BLNREP
Hansa Biopharma	IDEFIRIX
Holostem	HOLOCLAR
Incyte	MINJUVI PEMAZYRE
Intercept	OCALIVA
Ipsen	COMETRIQ
Janssen	SIRTURO
Kite Pharma	TECARTUS
Kyowa Kirin	CRYSVITA
Mylan	DOVPRELA
MYR (Gilead)	HEPCLUDEX
Novartis	ADAKVEO
Otsuka	DELTYBA
PTC Therapeutics	TRANSLARNA
Roche	POLIVY
Akcea (SOBI)	WAYLIVRA
	ADCETRIS
Takeda	NATPAR
	NINLARO

EXCEPTIONAL CIRCUMSTANCES	
Albireo	BYLVAY
Alexion	STRENSIQ
Amryt	MYALEPTA
BioMarin	BRINEURA
Chiesi	RAXONE LAMZEDE
Clinuvel	SCENESSE
EUSA Pharma	QARZIBA
Gentium	DEFITELIO
Laboratoires CTRS	ORPHACOL
Leadiant	QUENODESOXYCHOLIC ACID
Pfizer	VYNDAQEL
SFL Pharmaceuticals	OBILTOXAXIMAB
Stemline	ELZONRIS
Ultragenyx	MEPSEVII

## II. Current condition of the ODs' authorization status with European marketing authorization

ADDITIONAL MONITORING		ADDITIONAL MONITORING		ADDITIONAL MONITORING		ADDITIONAL MONITORING	
Abllynx/Sanofi	CABLIVI	Bioprojet	WAKIX	Intercept	OCALIVA	Chiesi	RAXONE
Advanced Accelerator Applications	LUTATHERA	bluebird bio	SKYSONA	Ipsen	COMETRIQ		BESPONSA
	SOMAKIT	Blueprin	ZYNTEGLO		XERMELO	Pfizer	DAURISMO
Alexion	STRENSIQ	Deciphera	AYVAKYT	Janssen	SIRTURO		MYLOTARG
	KANUMA	BMS/Celgene	QINLOCK		DARZALEX		VYNDAQEL
	ONPATTRO		IMNOVID	Kite Pharma	TECARTUS	PTC Therapeutics	TRANSLARNA
Alnylam	GIVLAARI	BMS	ABECMA		YESCARTA	Recordati Rare Diseases	ISTURISA
	OXLUMO		INREBIC	Kyowa Kirin	CRYSVITA	Rhythm	IMCIVREE
Amgen	BLINCYTO		REBLOZYL		POTELIGEO	Roche	GAZYVARO
Amicus	GALAFOLD	Chiesi	LAMZEDE	Laboratoires CTRS	ORPHACOL		POLIVY
Amryt	MYALEPTA	Clinuvel	SCENESSE			SFL Pharmaceuticals	OBILTOXAXIMAB
Astellas	XOSPATA	CSL	IDELVION	Leadiant	QUENODESOXYCHOLIC ACID	SOBI	ASPAVELI
	KOSELUGO	Dompe	OXERVATE				ALPROLIX
AstraZeneca	LUMOXITI	EUSA Pharma	QARZIBA	MediWound	NEXOBRID	AKCEA (SOBI)	TEGSEDI
		Gentium	DEFITELIO	MSD	PREVYMIS		WAYLIVRA
Novartis (Avexis)	ZOLGENSMA	Genzyme	CERDELGA			Stemline	ELZONRIS
Biogen	SPINRAZA		BLENREP	Mylan	DOVPRELA (Previously: PRETOMANID)	Shire (Takeda)	REVESTIVE
	BRINEURA	GlaxoSmithKline	ZEJULA				ALOFISEL
	PALYNZIQ	Hansa Biopharma	IDEFIRIX	MYR (Gilead)	HEPCLUDEX		ADCETRIS
BioMarin	VOXZOGO	Holostem	HOLOCLAR		KYMRIAH	Takeda	TAKHZYRO
	VIMIZIM	HRA Pharma Rare Diseases	KETOCONAZOLE	Novartis	LUXTURNA		NATPAR
		Immedica/SOBI	RAVICTI		ADAKVEO		NINLARO
			PEMAZYRE		RYDAPT	Ultragenyx	MEPSEVII
		Incyte	MINJUVI	Orchard Therapeutics	LIBMELDY		KAFTRIO
			ICLUSIG		STRIMVELIS	Vertex	SYMKEVI
				Otsuka	DELTYBA	Zogenix/MSD	FINTEPLA

## 12. Current condition of the ODs' authorization status with NC

ACCELERATED EVALUATION	
Albireo	BYLVAY
	KANUMA
Alexion	SOLIRIS
Alnylam	ONPATTRO
Amgen	KYPROLIS
Biogen	SPINRAZA
BioMarin	BRINEURA
Dompe	OXERVATE
Dr Falk Pharma	JORVEZA
EUSA Pharma	SYLVANT
Incyte	ICLUSIG
Janssen	DARZALEX
Roche	EVRYSDI
Santen	VERKAZIA
AKCEA (SOBI)	TEGSEDI
	TAKHZYRO
Takeda	VPRIV
Vertex	KALYDECO

CONDITIONAL AUTHORIZATION	
Novartis (Avexis)	ZOLGENSMA
AstraZeneca	KOSELUGO
GlaxoSmithKline	BLENREP
Hansa	IDEFIRIX
Holostem	HOLOCLAR
	PEMAZYRE
Incyte	MINJUVI
Intercept	OCALIVA
Ipsen	COMETRIQ
Janssen	SIRTURO
Kite Pharma	TECARTUS
Kyowa Kirin	CRYSVITA
MYR (Gilead)	HEPCLUDEX
Novartis	ADAKVEO
Otsuka	DELTYBA
PTC Therapeutics	TRANSLARNA
Roche	POLIVY
Akcea (SOBI)	WAYLIVRA
	ADCETRIS
Takeda	NATPAR
	NINLARO

EXCEPTIONAL CIRCUMSTANCES	
Albireo	BYLVAY
Alexion	STRENSIQ
Amryt	MYALEPTA
BioMarin	BRINEURA
	LAMZEDE
Chiesi	RAXONE
Clinuvel	SCENESSE
EUSA Pharma	QARZIBA
Laboratoires CTRS	ORPHACOL
Leadiant	QUENODESOXYCHOLIC ACID
Pfizer	VYNDAQEL
Ultragenyx	MEPSEVII

## 12. Current condition of the ODs' authorization status with NC

ADDITIONAL MONITORING		ADDITIONAL MONITORING		ADDITIONAL MONITORING		ADDITIONAL MONITORING	
Ablynx/Sanofi	CABLIVI	Bioprojet	WAKIX	Intercept	OCALIVA	Chiesi	RAXONE
Advanced Accelerator Applications	LUTATHERA	BMS/Celgene	IMNOVID	Ipsen	COMETRIQ	Pfizer	BESPONSA
	SOMAKIT	BMS	INREBIC		XERMELO		DAURISMO
Alexion	STRENSIQ		REBLOZYL	Janssen	SIRTURO		MYLOTARG
	KANUMA	Chiesi	DARZALEX	TECARTUS	VYNDAQEL		
Alnylam	ONPATTRO	Clinuvel	Kite Pharma	YESCARTA	PTC Therapeutics	TRANSLARNA	
	GIVLAARI	CSL	Kyowa Kirin	CRYSVITA	Recordati Rare Diseases	ISTURISA	
Amgen	OXLUMO	Dompe	Laboratoires CTRS	POTELIGEO	Roche	GAZYVARO	
	BLINCYTO	EUSA Pharma	ORPHACOL	QUENODESOXYCHOLIC ACID		POLIVY	
Amicus	GALAFOLD	Genzyme	Leadiant	NEXOBRID	SOBI	ASPAVELI	
Amryt	MYALEPTA	GlaxoSmithKline	MediWound	PREVYMIS	AKCEA (SOBI)	ALPROLIX	
Astellas	XOSPATA		ZEJULA	MSD		HEPCLUDEX	TEGSEDI
AstraZeneca	KOSELUGO	Hansa	MYR (Gilead)	KYMRIAH	Shire (Takeda)	WAYLIVRA	
Novartis (Avexis)	ZOLGENSMA	Holostem	Novartis	LUXTURNA		REVESTIVE	
Biogen	SPINRAZA	Immedica/SOBI		ADAKVEO	RYDAPT	Takeda	ALOFISEL
	BRINEURA	Incyte	LIBMELDY	DELTYBA	ADCETRIS		
BioMarin	PALYNZIQ		MINJUVI	Orchard Therapeutics	Orchard Therapeutics		TAKHZYRO
	VIMIZIM	PEMAZYRE	Otsuka	Otsuka	NATPAR		
						NINLARO	
						MEPSEVII	
						KAFTRIO	
						SYMKEVI	
						FINTEPLA	

# 13. Current condition of the ODs' authorization status included in NHS funding

CONDITIONAL AUTHORIZATION		
Laboratoy	Commercial Name	Funding date
Novartis (Avexis)	ZOLGENSMA	01/12/2021
Intercept	OCALIVA	1/03/2018
Kyowa Kirin	CRYSVITA	01/01/2021
Otsuka	DELTYBA	01/02/2018
Roche	POLIVY	01/09/2021
Takeda	ADCETRIS	01/08/2014

EXCEPTIONAL CIRCUMSTANCES		
Laboratoy	Commercial Name	Funding date
Laboratoires CTRS	ORPHACOL	01/11/2015
Leadiant	QUENODESOXYCHOLIC ACID	01/12/2021
Pfizer	VYNDAQEL	01/12/2013
Ultragenyx	MEPSEVII	01/07/2021

ACCELERATED EVALUATION		
Laboratoy	Commercial Name	Funding date
Alexion	KANUMA	01/11/2017
	SOLIRIS	15/01/2008
Alnylam	ONPATTRO	01/05/2020
Amgen	KYPROLIS	01/11/2016
Biogen	SPINRAZA	01/03/2018
Eusa Pharma	SYLVANT	01/05/2016
Incyte	ICLUSIG	01/07/2017
Janssen	DARZALEX	01/12/2016
Takeda	VPRIV	01/03/2011
	TAKHZYRO	01/03/2021
Akcea (SOBI)	TEGSEDI	01/05/2020
Vertex	KALYDECO	01/08/2014

# 13. Current condition of the ODS' authorization status included in NHS funding



ADDITIONAL MONITORING		
Laboratoy	Commercial Name	Fecha Financiación
Ablynx/Sanofi	CABLIVI	01/07/2021
Advanced Accelerator Applications	LUTATHERA	01/04/2019
	SOMAKIT	01/04/2018
Alexion	KANUMA	01/11/2017
Alnylam	GIVLAARI	01/09/2021
	ONPATTRO	01/05/2020
Amicus	GALAFOLD	01/02/2018
Novartis (Avexis)	ZOLGENSMA	01/12/2021
Biogen	SPINRAZA	01/03/2018

ADDITIONAL MONITORING		
Laboratoy	Commercial Name	Fecha Financiación
Bioprojet	WAKIX	01/11/2020
BMS/Celgene	IMNOVID	01/07/2014
CSL	IDELVION	01/03/2020
Genzyme	CERDELGA	01/01/2017
GlaxoSmith Kline	ZEJULA	01/08/2019
Immedica/S OBI	RAVICTI	01/12/2017
Incyte	ICLUSIG	01/07/2017

ADDITIONAL MONITORING		
Laboratoy	Commercial Name	Funding date
Intercept	OCALIVA	01/03/2018
Janssen	DARZALEX	01/12/2016
Kite Pharma	YESCARTA	01/07/2019
Kyowa Kirin	CRYSVITA	01/01/2021
	POTELIGEO	01/07/2021
Laboratoire s CTRS	ORPHACOL	01/11/2015
Leadiant	QUENODESOXYCHOLIC ACID	01/12/2021
MSD	PREVYMIS	01/08/2021
	KYMRIAH	01/01/2019
Novartis	LUXTURNA	01/05/2021
Otsuka	RYDAPT	01/04/2019
	DELTYBA	01/02/2018

ADDITIONAL MONITORING		
Laboratoy	Commercial Name	Funding date
Pfizer	BESPONSA	01/07/2019
	MYLOTARG	01/07/2019
Roche	VYNDAQEL	01/12/2013
	GAZYVARO	01/11/2015
SOBI	POLIVY	01/09/2021
	ALPROLIX	01/03/2020
Akcea (SOBI)	TEGSEDI	01/05/2020
Takeda (Shire)	REVESTIVE	01/10/2017
	ALOFISEL	01/09/2019
Takeda	ADCETRIS	01/08/2014
	TAKHZYRO	01/03/2021
Ultragenyx	MEPSEVII	01/07/2021
Vertex	KAFTRIO	01/12/2021
	SYMKEVI	01/11/2019



# 14. Current condition of the ODs' authorization status not included in NHS funding

CONDITIONAL AUTHORIZATION		
Laboratoy	Commercial Name	NC year
AstraZeneca	KOSELUGO	2021
Glaxosmithkline	BLENREP	2020
Holostem	HOLOCLAR	2015
Incyte	PEMAZYRE	2021
	MINJUVI	2021
Ipsen	COMETRIQ	2015
Janssen	SIRTURO	2014
Kite Pharma	TECARTUS	2020
Novartis	ADAKVEO	2021
Ptc Therapeutics	TRANSLARNA	2014
Akcea (SOBI)	WAYLIVRA	2020
Takeda	NATPAR	2018
	NINLARO	2017

EXCEPTIONAL CIRCUMSTANCES		
Laboratoy	Commercial Name	NC year
Albireo	BYLVAY	2021
Alexion	STRENSIQ	2015
Amryt	MYALEPTA	2018
Biomarin	BRINEURA	2019
Chiesi	LAMZEDE	2018
	RAXONE	2018
Clinuvel	SCENESSE	2017
Eusa Pharma	QARZIBA	2018

ACCELERATED EVALUATION		
Laboratoy	Commercial Name	NC year
Albireo	BYLVAY	2021
Biomarin	BRINEURA	2019
Dompe	OXERVATE	2017
Dr. Falk Pharma	JORVEZA	2018
Roche	EVRYSDI	2021
Santen	VERKAZIA	2019

# 14. Current condition of the ODs' authorization status not included in NHS funding

ADDITIONAL MONITORING		
Laboratoy	Commercial Name	NC year
Alexion	STRENSIQ	2015
Alnylam	OXLUMO	2020
Amgen	BLINCYTO	2015
Amryt	MYALEPTA	2018
Astellas	XOSPATA	2019
AstraZeneca	KOSELUGO	2021
	BRINEURA	2019
Biomarin	PALYNZIQ	2019
	VIMIZIM	2015

ADDITIONAL MONITORING		
Laboratoy	Commercial Name	NC year
BMS	INREBIC	2021
	REBLOZYL	2020
Chiesi	LAMZEDE	2018
Clinuvel	SCENESSE	2017
Dompe	OXERVATE	2017
Eusa Pharma	QARZIBA	2018
Glaxosmithkline	BLENREP	2020
Holostem	HOLOCLAR	2015
Incyte	MINJUVI	2021
	PEMAZYRE	2021
Ipsen	COMETRIQ	2015
	XERMELO	2017

ADDITIONAL MONITORING		
Laboratoy	Commercial Name	NC year
Janssen	SIRTURO	2014
Kite Pharma	TECARTUS	2020
Mediwound	NEXOBRID	2014
Novartis	ADAKVEO	2021
Orchard Therapeutics	LIBMELDY	2021
Pfizer	DAURISMO	2020
Ptc Therapeutics	TRANSLARNA	2014

ADDITIONAL MONITORING		
Laboratoy	Commercial Name	NC year
Recordati Rare Diseases	ISURISA	2021
Akcea (SOBI)	WAYLIVRA	2020
SOBI	ASPAVELI	2021
Takeda	NATPAR	2018
	NINLARO	2017
Zogenix/MSD	FINTEPLA	2021

# 15. ODs funded during 2021, situation in Europe ( 1/3)

Commercial Name	Laboratoy	NHS funding date	Number of European countries where it has received a favourable opinion on funding	European countries where it has received a favourable opinion on funding	Date on which it received a favourable opinion on funding
1. CRYSVITA	Kyowa Kirin	01/01/2021	13	<ol style="list-style-type: none"> <li>Germany</li> <li>Belgium</li> <li>Denmark</li> <li>Slovakia</li> <li>Slovenia</li> <li>Finland</li> <li>France</li> <li>Italy</li> <li>Luxembourg</li> <li>Norway</li> <li>Romania</li> <li>Sweden</li> <li>UK</li> </ol>	<ol style="list-style-type: none"> <li>27/08/2019</li> <li>-</li> <li>20/11/2019</li> <li>-</li> <li>-</li> <li>-</li> <li>23/01/2019</li> <li>27/08/2019</li> <li>-</li> <li>-</li> <li>-</li> <li>14/02/2019</li> <li>10/10/2018</li> </ol>
2. TAKHZYRO	Takeda	01/03/2021	14	 <ol style="list-style-type: none"> <li>Bulgaria</li> <li>Denmark</li> <li>Slovakia</li> <li>Slovenia</li> <li>Finland</li> <li>France</li> <li>Germany</li> <li>Ireland</li> <li>Italy</li> <li>Liechtenstain</li> <li>Norway</li> <li>Poland</li> <li>Switzerland</li> <li>UK</li> </ol>	<ol style="list-style-type: none"> <li>-</li> <li>22/01/2020</li> <li>-</li> <li>-</li> <li>-</li> <li>05/06/2019</li> <li>01/08/2019</li> <li>26/06/2020</li> <li>27/03/2020</li> <li>-</li> <li>-</li> <li>-</li> <li>-</li> <li>16/10/2019</li> </ol>
3. LUXTURNA	Novartis	01/05/2021	9	<ol style="list-style-type: none"> <li>Belgium</li> <li>Croatia</li> <li>Denmark</li> <li>France</li> <li>Germany</li> <li>Ireland</li> <li>Italy</li> <li>Netherlands</li> <li>UK</li> </ol>	<ol style="list-style-type: none"> <li>-</li> <li>-</li> <li>23/04/2020</li> <li>03/04/2019</li> <li>17/10/2019</li> <li>18/09/2020</li> <li>22/12/2020</li> <li>17/02/2020</li> <li>09/10/2018</li> </ol>
4. CABLIVI	Ablynx/Sanofi	01/07/2021	11	<ol style="list-style-type: none"> <li>Belgium</li> <li>Slovakia</li> <li>Slovenia</li> <li>France</li> <li>Germany</li> <li>Netherlands</li> <li>Italy</li> <li>Luxembourg</li> <li>Czech Republic</li> <li>Romania</li> <li>UK</li> </ol>	<ol style="list-style-type: none"> <li>-</li> <li>-</li> <li>-</li> <li>20/02/2019</li> <li>22/03/2019</li> <li>-</li> <li>03/01/2020</li> <li>-</li> <li>-</li> <li>-</li> <li>-</li> </ol>
5. MEPSEVII	Ultragenyx	01/07/2021	2	<ol style="list-style-type: none"> <li>Germany</li> <li>Italy</li> </ol>	<ol style="list-style-type: none"> <li>22/03/2019</li> <li>30/08/2021</li> </ol>



# 15. ODs funded during 2021, situation in Europe (2/3)

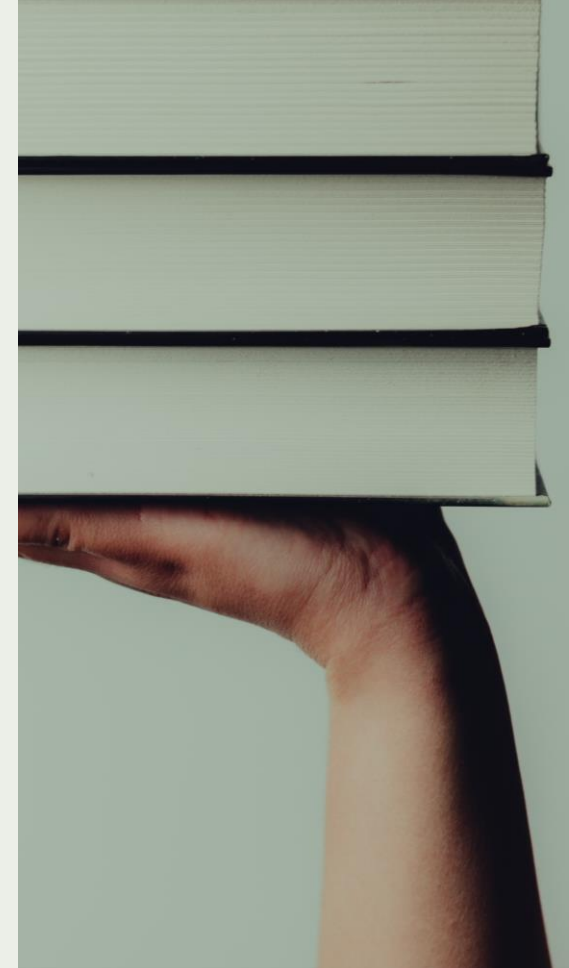
Commercial Name	Laboratoy	NHS funding date	Number of European countries where it has received a favourable opinion on funding	European countries where it has received a favourable opinion on funding	Date on which it received a favourable opinion on funding
6. POTELIGEO	Kyowa Kirin	01/07/2021	6	<ol style="list-style-type: none"> <li>1. Belgium</li> <li>2. Slovenia</li> <li>3. France</li> <li>4. Germany</li> <li>5. Italy</li> <li>6. UK</li> </ol>	<ol style="list-style-type: none"> <li>1. -</li> <li>2. -</li> <li>3. 17/07/2019</li> <li>4. 03/12/2020</li> <li>5. 24/11/2020</li> <li>6. 15/12/2021</li> </ol>
7. PREVYMIS	MSD	01/08/2021	21	<ol style="list-style-type: none"> <li>1. Belgium</li> <li>2. Croatia</li> <li>3. Denmark</li> <li>4. Slovenia</li> <li>5. Finland</li> <li>6. France</li> <li>7. Germany</li> <li>8. Hungary</li> <li>9. Italy</li> <li>10. Ireland</li> <li>11. Netherlands</li> <li>12. Latvia</li> <li>13. Liechtenstein</li> <li>14. Luxembourg</li> <li>15. Norway</li> <li>16. Portugal</li> <li>17. Czech Republic</li> <li>18. Romania</li> <li>19. Sweden</li> <li>20. Switzerland</li> <li>21. UK</li> </ol>	<ol style="list-style-type: none"> <li>1. -</li> <li>2. -</li> <li>3. 15/08/2018</li> <li>4. -</li> <li>5. -</li> <li>6. 05/09/2018</li> <li>7. 02/08/2018</li> <li>8. -</li> <li>9. 03/09/2018</li> <li>10. -</li> <li>11. 27/11/2018</li> <li>12. -</li> <li>13. -</li> <li>14. -</li> <li>15. -</li> <li>16. 30/12/2019</li> <li>17. -</li> <li>18. -</li> <li>19. -</li> <li>20. -</li> <li>21. 31/07/2019</li> </ol>
8. TREPULMIX	SciPharm/AOP Orphan	01/08/2021	3	<ol style="list-style-type: none"> <li>1. Alemania</li> <li>2. Latvia</li> <li>3. Romania</li> </ol>	<ol style="list-style-type: none"> <li>1. -</li> <li>2. -</li> <li>3. 13/09/2021</li> </ol>
9. EPIDYOLEX	Gw Pharma	01/09/2021	8	<ol style="list-style-type: none"> <li>1. Germany</li> <li>2. Austria</li> <li>3. Denmark</li> <li>4. Finland</li> <li>5. Italy</li> <li>6. Norway</li> <li>7. Sweden</li> <li>8. UK</li> </ol>	<ol style="list-style-type: none"> <li>1. 02/04/2020</li> <li>2. -</li> <li>3. -</li> <li>4. -</li> <li>5. -</li> <li>6. -</li> <li>7. -</li> <li>8. 18/12/2019</li> </ol>
10. GIVLAARI	Alnylam	01/09/2021	7	<ol style="list-style-type: none"> <li>1. Belgium</li> <li>2. Slovenia</li> <li>3. France</li> <li>4. Germany</li> <li>5. Italy</li> <li>6. Luxembourg</li> <li>7. Netherlands</li> </ol>	<ol style="list-style-type: none"> <li>1. -</li> <li>2. -</li> <li>3. 24/03/2021</li> <li>4. 15/10/2020</li> <li>5. 12/01/2021</li> <li>6. -</li> <li>7. 02/02/2021</li> </ol>



## 15. ODs funded during 2021, situation in Europe (3/3)

Commercial Name	Laboratoy	NHS funding date	Number of European countries where it has received a favourable opinion on funding	European countries where it has received a favourable opinion on funding	Date on which it received a favourable opinion on funding
11. POLIVY	Roche	01/09/2021	7	1. Germany 2. Bulgaria 3. Slovenia 4. Ireland 5. Latvia 6. Czech Republic 7. UK	1. 20/08/2020 2. - 3. - 4. 16/02/2021 5. - 6. - 7. 23/09/2020
12. QUENODESOXYCHOLIC ACID	Leadiant	01/12/2021	6	1. Germany 2. France 3. Netherlands 4. Italy 5. Norway 6. UK	1. - 2. 11/07/2018 3. 22/11/2018 4. - 5. - 6. -
13. KAFTRIO	Vertex	01/12/2021	13	1. Bulgaria 2. Croatia 3. Slovakia 4. Slovenia 5. Finland 6. France 7. Germany 8. Italy 9. Ireland 10. Luxembourg 11. Norway 12. Portugal 13. UK	1. - 2. - 3. - 4. - 5. - 6. 18/11/2020 7. 18/02/2021 8. 07/01/2021 9. - 10. - 11. - 12. 21/07/2021 13. -
14. ZOLGENSMA	Novartis (Avexis)	01/12/2021	13	1. Germany 2. Belgium 3. Croatia 4. Denmark 5. Finland 6. Slovenia 7. Ireland 8. Italy 9. Latvia 10. Portugal 11. Czech Republic 12. Romania 13. UK	1. 04/11/2021 2. - 3. - 4. 26/05/2021 5. 30/10/2020 6. - 7. 13/05/2020 8. 10/03/2021 9. - 10. 13/10/2021 11. - 12. 07/12/2021 13. 07/07/2021

1. Positive orphan designation drugs of the European Medicines Agency: [https://www.ema.europa.eu/en/medicines/download-medicine-data#rare-disease-\(orphan\)-designations-section](https://www.ema.europa.eu/en/medicines/download-medicine-data#rare-disease-(orphan)-designations-section)
2. Community Register of Orphan Drugs : [http://ec.europa.eu/health/documents/community-register/html/reg\\_od\\_act.htm?sort=a](http://ec.europa.eu/health/documents/community-register/html/reg_od_act.htm?sort=a)
3. Orphan Drugs authorized by AEMPS: <https://cima.aemps.es/cima/publico/home.html>
4. Orphan Drugs NHS funded (Nomenclátor): <https://www.mscbs.gob.es/profesionales/farmacia/home.htm>
5. Interministerial Commission on Drug Prices Agreements: <https://www.mscbs.gob.es/profesionales/farmacia/CIPMyPS.htm>



- EMA: European Medicines Agency
- OD: Orphan Designation
- MA: European Marketing Authorization by Centralized Procedure
- UE: European Union
- NC: National Code
- NHS: National Health System
- AEMPS: Spanish Agency of Medicines and Health Products
- ODs: Orphan Drugs
- CHMP: Committee for Medicinal Products for Human Use
- COMP: Committee for Orphan Medicinal Products and Orphan Products
- CIPM: Interministerial Drug Pricing Commission



