Market ACCESS REPORT

to orphan medicinal products (OMPs) in Spain *

JAN – APRIL 2026

aelmhu

elaborado por:
bio innova Consulting

*Analysis of all OMPs with a brand name that have a valid European orphan designation as of April 30, 2026.

Table of contents

1 Methodology
2 Orphan Medicinal Products in the European Union and Spain
3 Orphan Medicinal Products in Spain
4 Orphan Medicinal Products reimbursed between January and April of 2026
5 Orphan Medicinal Products not reimbursed
6 Advanced Therapy Medicinal Products for rare diseases
7 Medicinal products indicated for rare diseases that have lost orphan designation
8 Summary: key facts
9 Bibliography
10 Acronyms

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Methodology

01 Of the more than 3,000 active substances with orphan designation (OD) by the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA), those active substances that have a valid orphan designation as of April 30, 2026 (more than 2,000) have been identified. The OD is granted during the early stages of medicines research, so among the active substances with a valid OD, those with a brand name (more than 250) are identified in each report. Duplicates with multiple indications are removed. After removing these duplicates, **230 orphan medicinal products (OMPs) with a valid OD as of April 30, 2026**, and with a brand name are obtained.

02 Next, it has been analyzed which of these orphan medicinal products have been withdrawn (or have not yet been included) from the European Commission's Community Register of Orphan Medicinal Products for marketing in the European Union (n=67). As a result, **the number of orphan medicinal products with marketing authorization (MA) as of April 30, 2026**, in the European Union is obtained (n=163).

03 The third step has been to identify which of **those orphan medicinal products with a brand name that have a marketing authorization in the European Union have a National Code (NC)** from the Spanish Agency of Medicines and Medical Devices (AEMPS) in Spain (n=141).

04 Finally, **the OMPs and their reimbursement dates by Spain's National Health System (SNS)** were identified according to the information collected in the Search Tool for Information on the Reimbursement Status of Medications (BIFIMED) of the Ministry of Health (n=106). The first registration date has been taken as the reimbursement date.

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Notes:

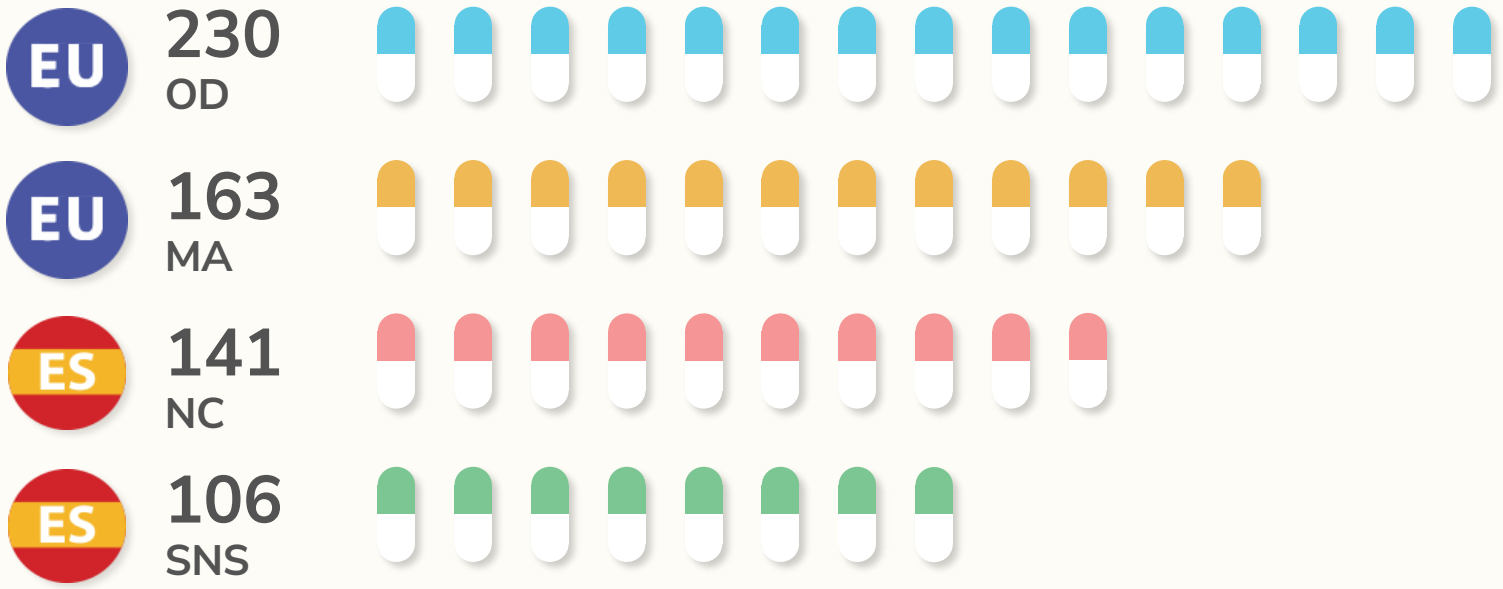
1. Because the data cannot be obtained from any public source, this document does not include those OMPs that are available in our country under specific circumstances and special authorizations provided for in Royal Decree 1015/2009 (compassionate use, use under conditions other than those authorized, or access to foreign medicines).
2. The company listed for each OMP throughout this report generally corresponds to the marketing authorization holder; however, in the case of reimbursed medicines, the company supplying the National Health System is identified, provided that it is not a pharmaceutical distributor.
3. Likewise, the values represented in the charts are usually shown rounded to whole numbers to facilitate understanding. Exceptionally, decimals have been included when the sum of rounded percentages results in values below or above 100.

Orphan Medicinal Products

European Union and Spain



Main indicators 2026*



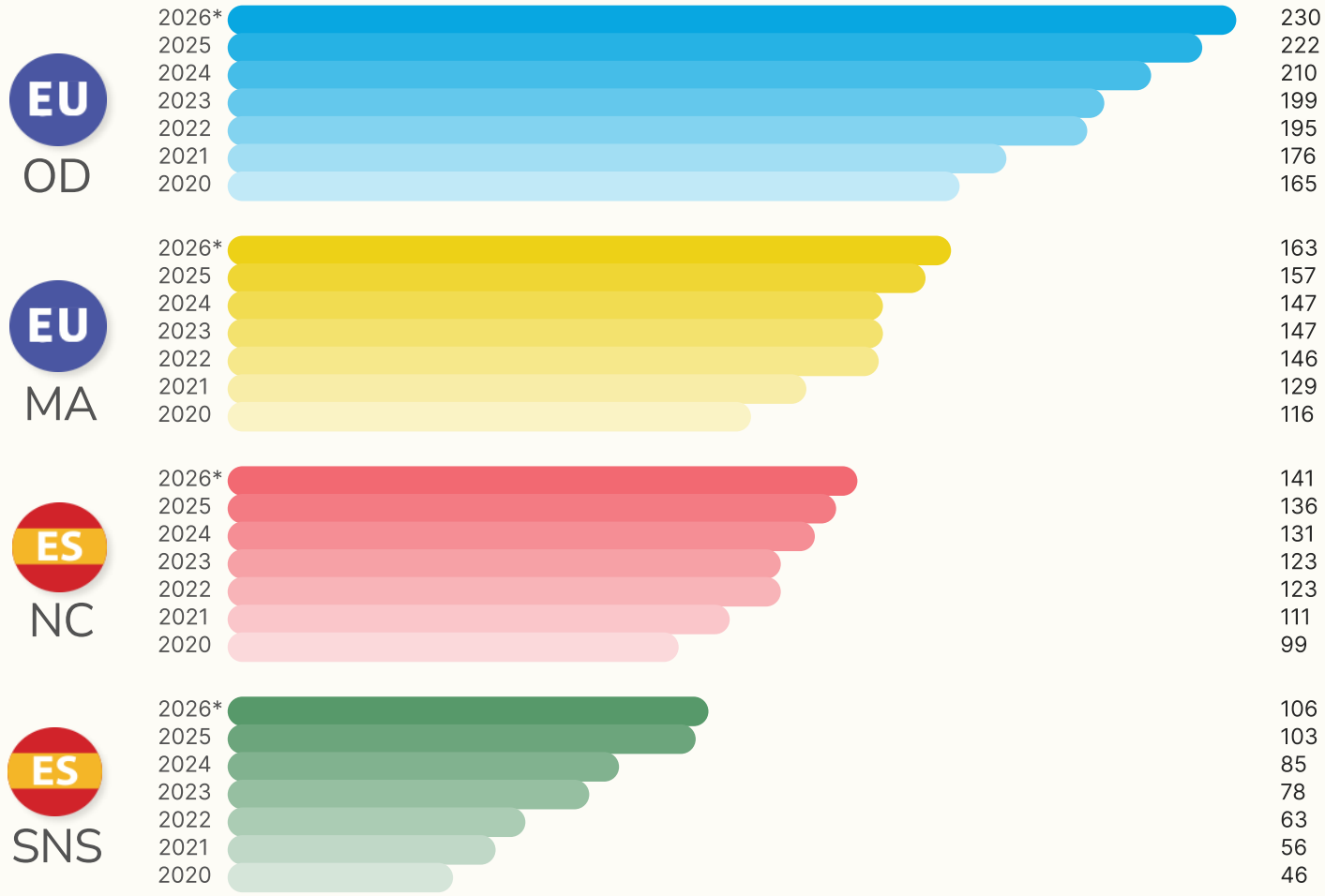
Notes:

OD: medicines with orphan designation granted by the EMA.
MA: orphan medicinal products with marketing authorization from the European Commission.
NC: orphan medicinal products with National Code in Spain.
SNS: orphan medicinal products reimbursed by the National Health System.

*Data accumulated until April 30, 2026.

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*Evolution of the indicators 2020-2026**

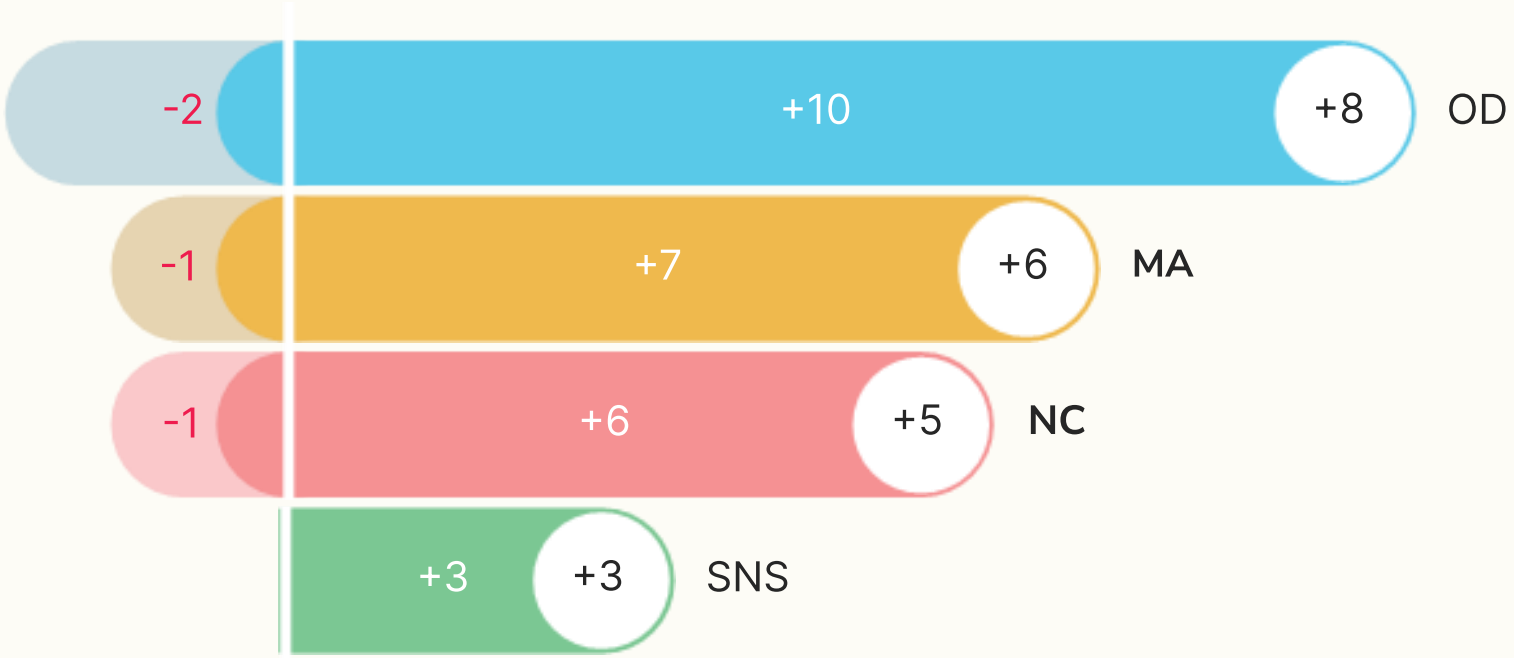


Note 1: Due to an update error in the EMA database, HOLOCLAR, marketed by the company Holostem (which obtained an extension of its MA for 2 years on October 23, 2025), was not accounted for in the OD, MA, and NC sections in the previous report.
Note 2: Due to an update error in the CIMA database, ENJAYMO, marketed by the company Sanofi (which obtained NC on December 29, 2025), was not accounted for in the NC section of the previous report.

*Data accumulated until April 30, 2026.

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*Variation of indicators between 2025 and 2026**



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*Data accumulated until April 30, 2026.

Detail of the variation of orphan designations

EU

OD

2025 vs. 2026*	OMPs	Company	Comments
+ 10 OMPs obtain OD	DAYBU IMDYLLTRA JOENJA KYGEVVI OJEMDA PALSONIFY REDEMPLO VIOKAT XOLREMDI ZEPZELCA	Acadia Pharmaceuticals Amgen Pharming Technologies UCB Pharma Ipsen Crinetics Pharmaceuticals Arrowhead Pharmaceuticals Soleno Therapeutics X4 Pharmaceuticals PharmaMar	CHMP 26/02/2026 CHMP 26/03/2026 CHMP 26/03/2026 MA 26/03/2026 CHMP 26/02/2026 MA 23/04/2026 CHMP 23/04/2026 No MA 23/04/2026 CHMP 26/02/2026 CHMP 26/03/2026
- 2 OMPs lose OD	HETRONIFLY SOHONOS	Accord Ipsen	Lost OD Lost OD

*Data accumulated until April 30, 2026.

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Detail of the variation of marketing authorizations

	2025 vs. 2026*	OMPs	Company	Comments
EU MA	+ 7 OMPs obtain MA	AQNEURSA KYGEVVI OJEMDA PALSONIFY REZUROCK WASKYRA XOLREMDI	IntraBio UCB Pharma Ipsen Crinetics Pharmaceuticals Sanofi Fondazione Telethon X4 Pharmaceuticals	MA 19/01/2026 MA 26/03/2026 MA 20/04/2026 MA 23/04/2026 MA 24/03/2026 MA 09/01/2026 MA 27/04/2026
	- 1 OMP loses OD	HETRONIFLY	Accord	Lost OD

*Data accumulated until April 30, 2026.

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Detail of the variation of national codes

	2025 vs. 2026*	OMPs	Company	Comments
ES NC	+ 6 OMPs obtain NC	EZMEKLY OGSIVEO REZUROCK VORANIGO WAYRILZ ZYNYZ	SpringWorks Therapeutics Springworks Therapeutics Sanofi Servier Sanofi Incyte	NC 18/02/2026 NC 02/01/2026 NC 31/03/2026 NC 02/01/2026 NC 17/04/2026 NC 02/03/2026
	- 1 OMP loses OD	HETRONIFLY	Accord	Lost OD

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*Data accumulated until April 30, 2026.

Detail of the variation of reimbursed OMPs

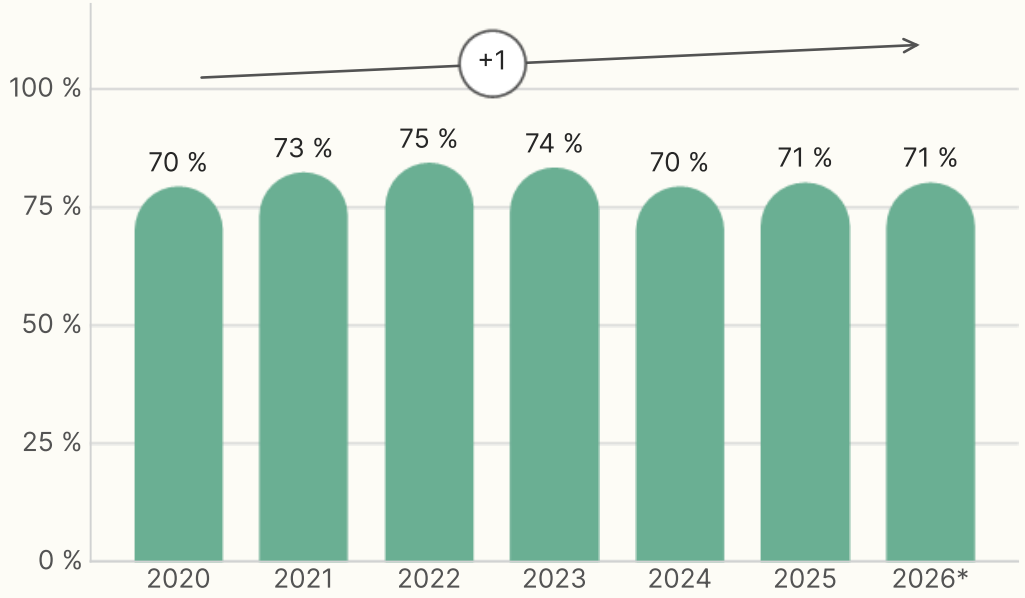
	2025 vs. 2026*	OMPs	Company	Comments
SNS	+ 3 OMPs obtain reimbursement	AGAMREE FILSPARI SKYCLARYS	Santhera Pharmaceuticals Vifor Biogen	SNS 01/04/2026 SNS 01/04/2026 SNS 01/03/2026
	0 OMP lose OD, MA and/or NC	-	-	-

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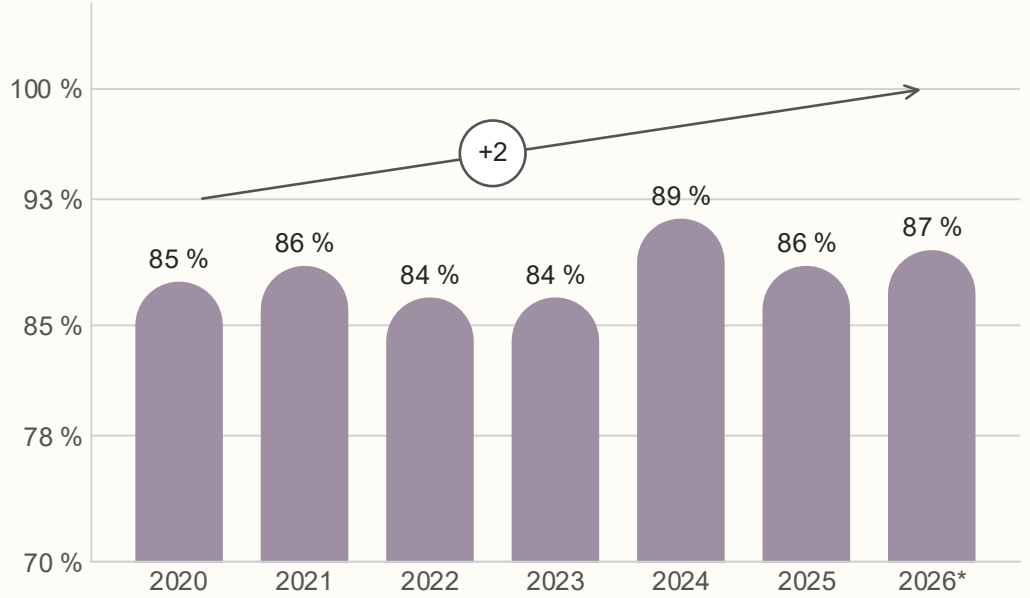
*Data accumulated until April 30, 2026.

Comparison between the indicators

% of OMPs with OD and MA



% of OMPs with MA and NC

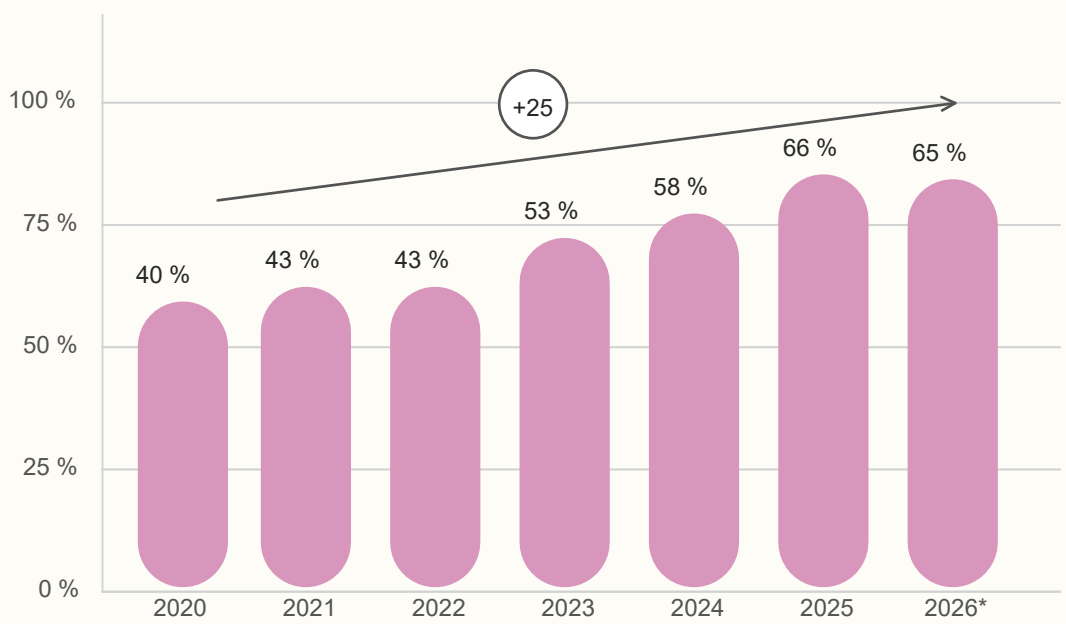


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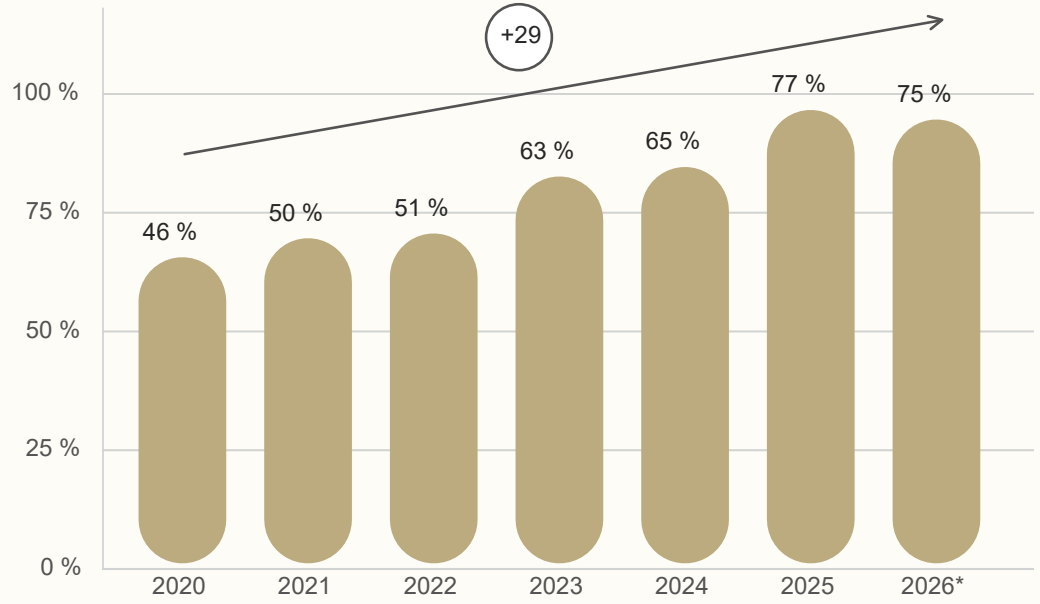
*Data accumulated until April 30, 2026.

Comparison between the indicators

% of OMPs with MA and reimbursed by the SNS



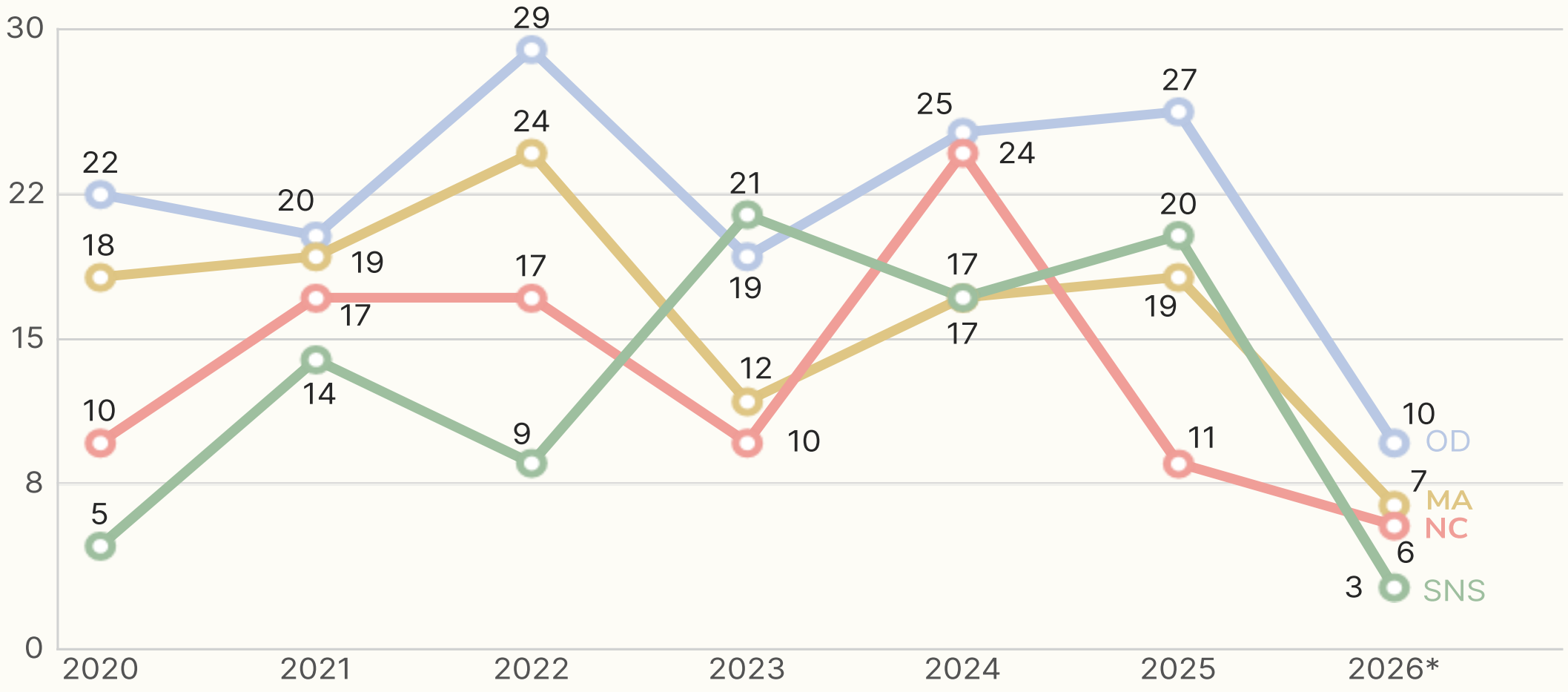
% of OMPs with NC and reimbursed by the SNS



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*Data accumulated up to April 30, 2026.

Summary of indicators by year

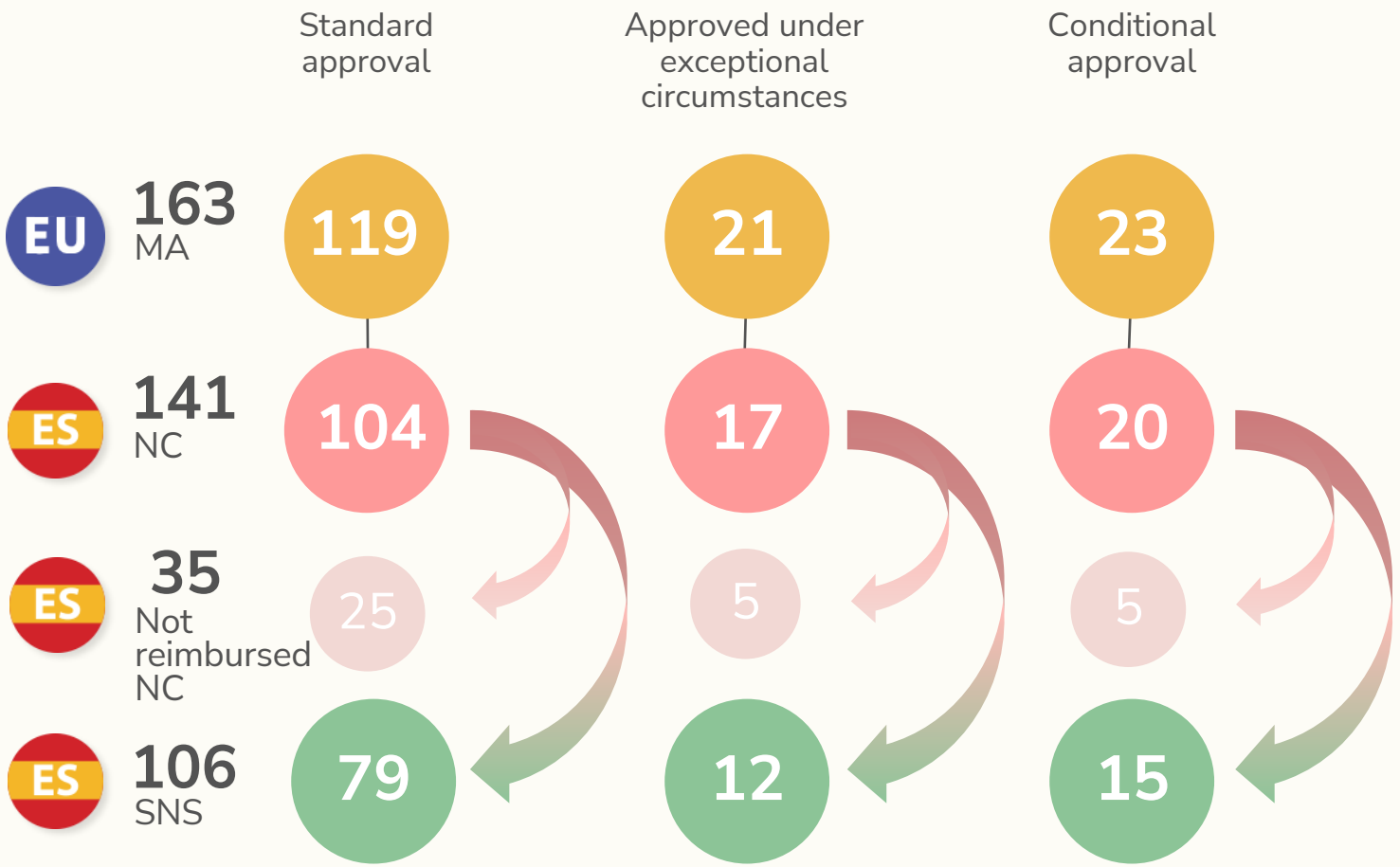


Note 1: Due to an update error in the EMA database, HOLOCLAR, marketed by the company Holostem (which obtained an extension of its OD for 2 years on October 23, 2025), was not accounted for in the OD, MA, and NC sections in the previous report.
Note 2: Due to an update error in the CIMA database, ENJAYMO, marketed by the company Sanofi (which obtained NC on December 29, 2025), was not accounted for in the NC section in the previous report.

*Data accumulated up to April 30, 2026.

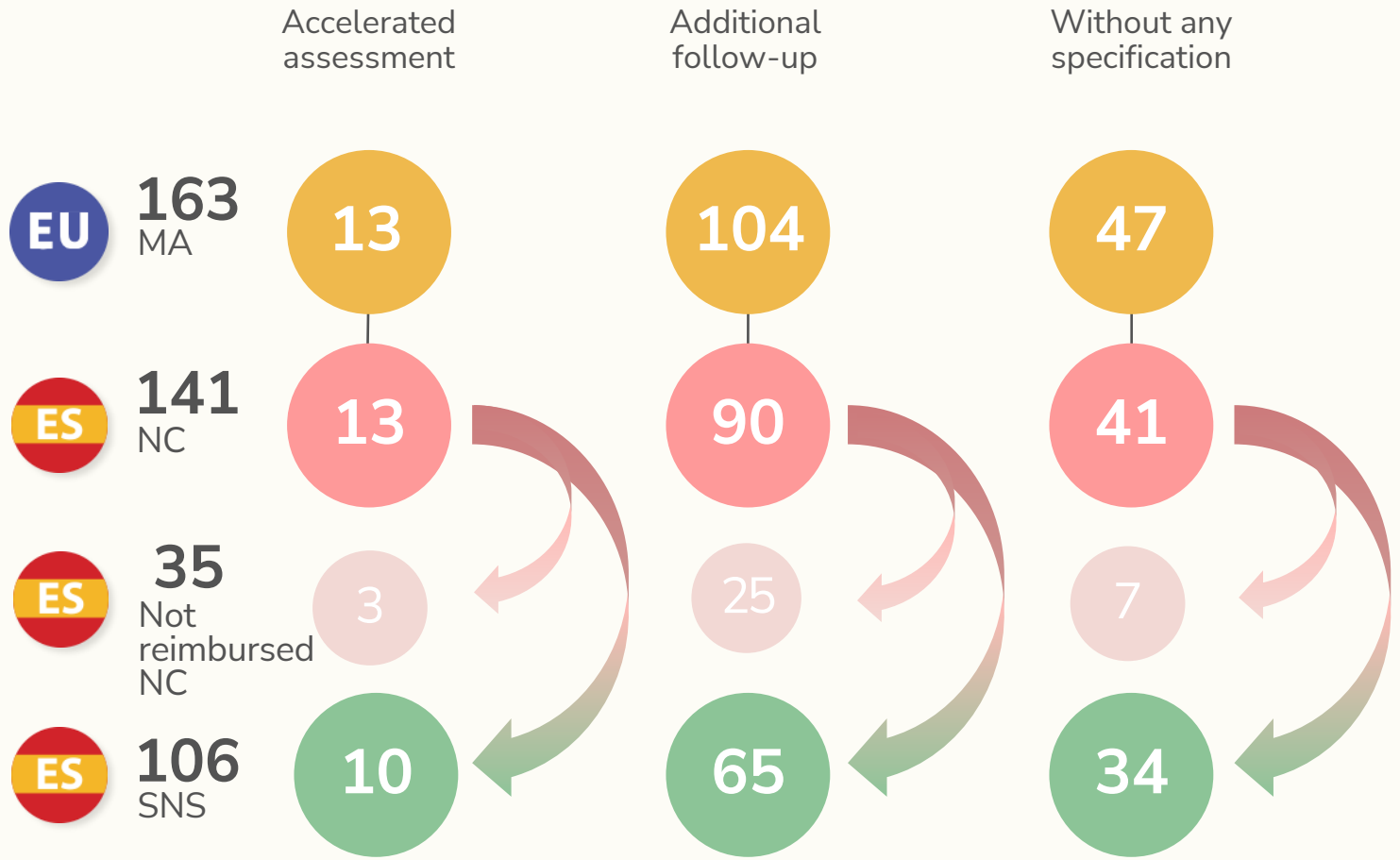
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Type of approval for orphan medicinal products



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*Special conditions associated with the approval of OMPs**

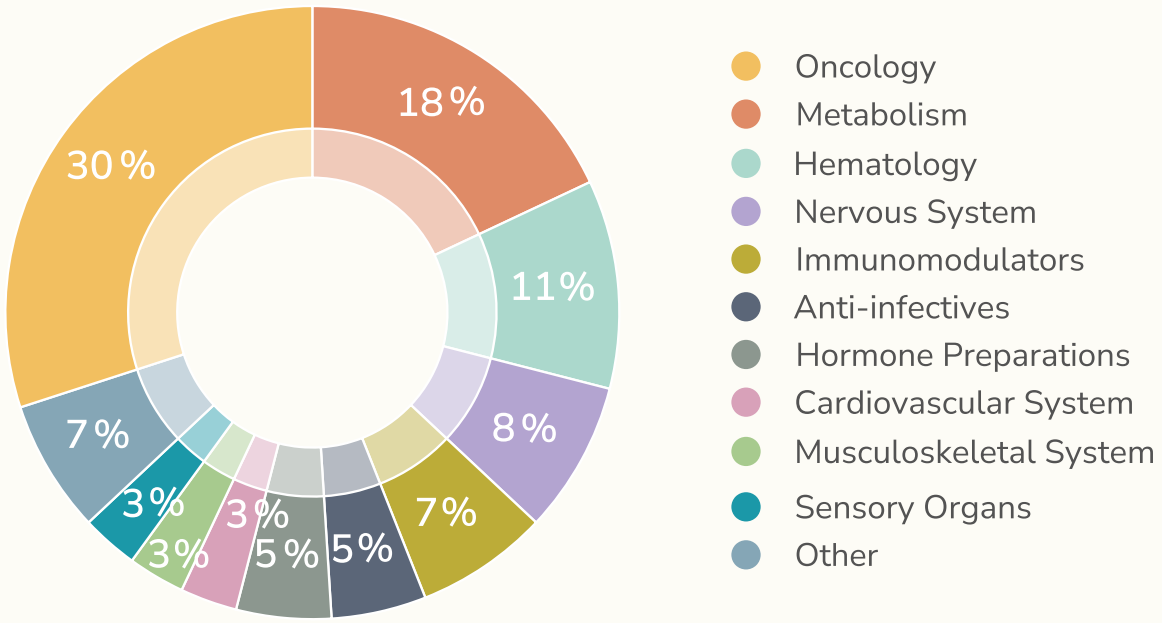


*The same orphan medicinal product may be subject to more than one special condition simultaneously. For this reason, the sum of “accelerated assessment,” “additional monitoring,” and “no specification” does not necessarily match the total OMPs.

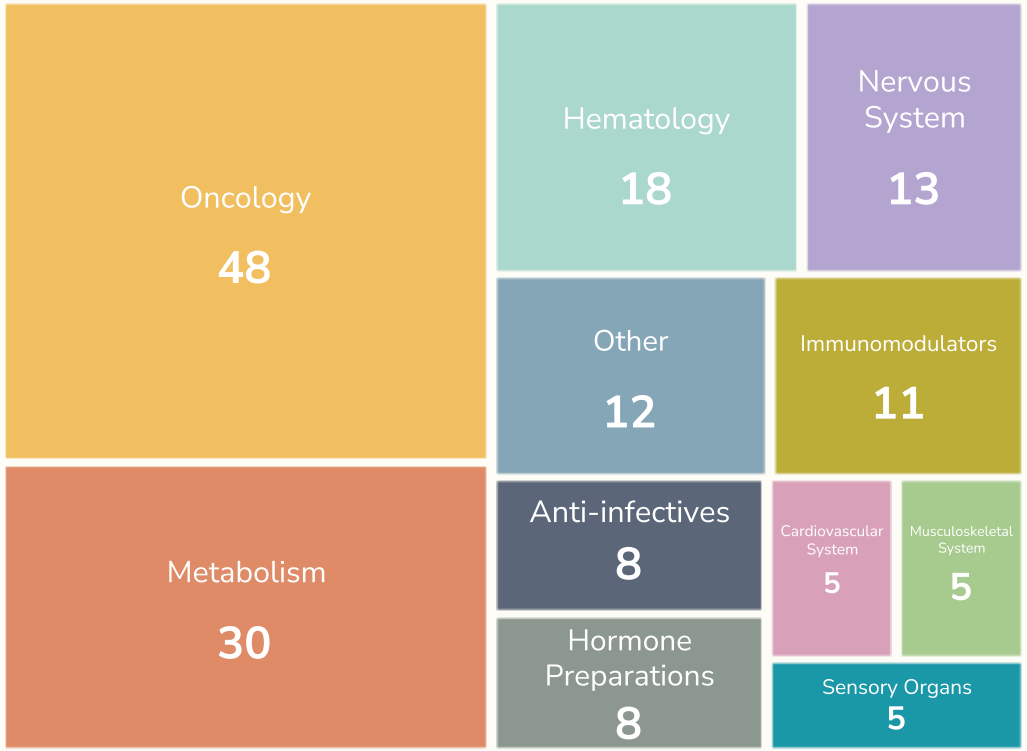
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Orphan Medicinal Products with marketing authorization

By therapeutic area

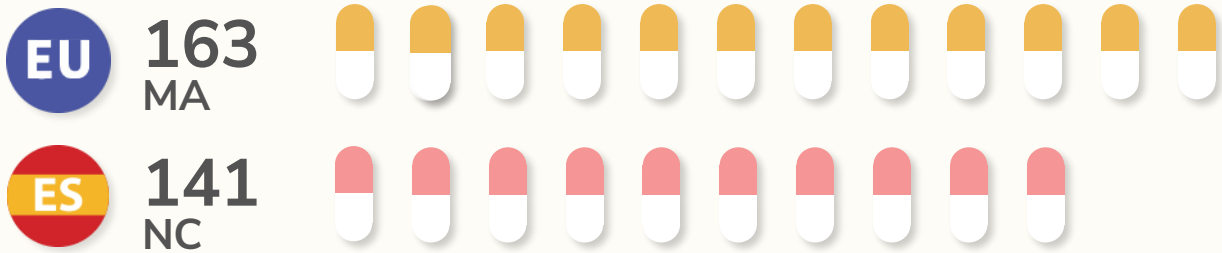


Absolute values



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Orphan Medicinal Products with MA vs. NC



As of April 30, 2026, 87% of the OMPs that have MA have a National Code in Spain.

22 medicines still do not have NC.

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Detailed information of the 22 OMPs with MA without NC (1/2)

	Brand name	Company	MA Date	Waiting months (MA – 30/04/2026)	Therapeutic area	ATMP
1	AQNEURSA	IntraBio	19/01/2026	3	Nervous system	
2	ARIKAYCE	Insmed	27/10/2020	67	Anti-infectives	
3	DOVPRELA	Mylan	31/07/2020	70	Anti-infectives	
4	EKTERLY	Kalvista	17/09/2025	8	Hematology	
5	EMCITATE	Rare Thyroid Therapeutics International	12/02/2025	15	Hematology	
6	HYFTOR	PlusUltra	15/05/2023	36	Dermatology	
7	KYGEVVI	UCB Pharma	26/03/2026	1	Metabolism	
8	MAAPLIV	Recordati	28/07/2025	9	Metabolism	
9	NULIBRY	Comharsa	15/09/2022	44	Metabolism	
10	OJEMDA	Ipsen	20/04/2026	0	Oncology	
11	PALSONIFY	Crinetics Pharmaceuticals	23/04/2026	0	Hormone Preparations	

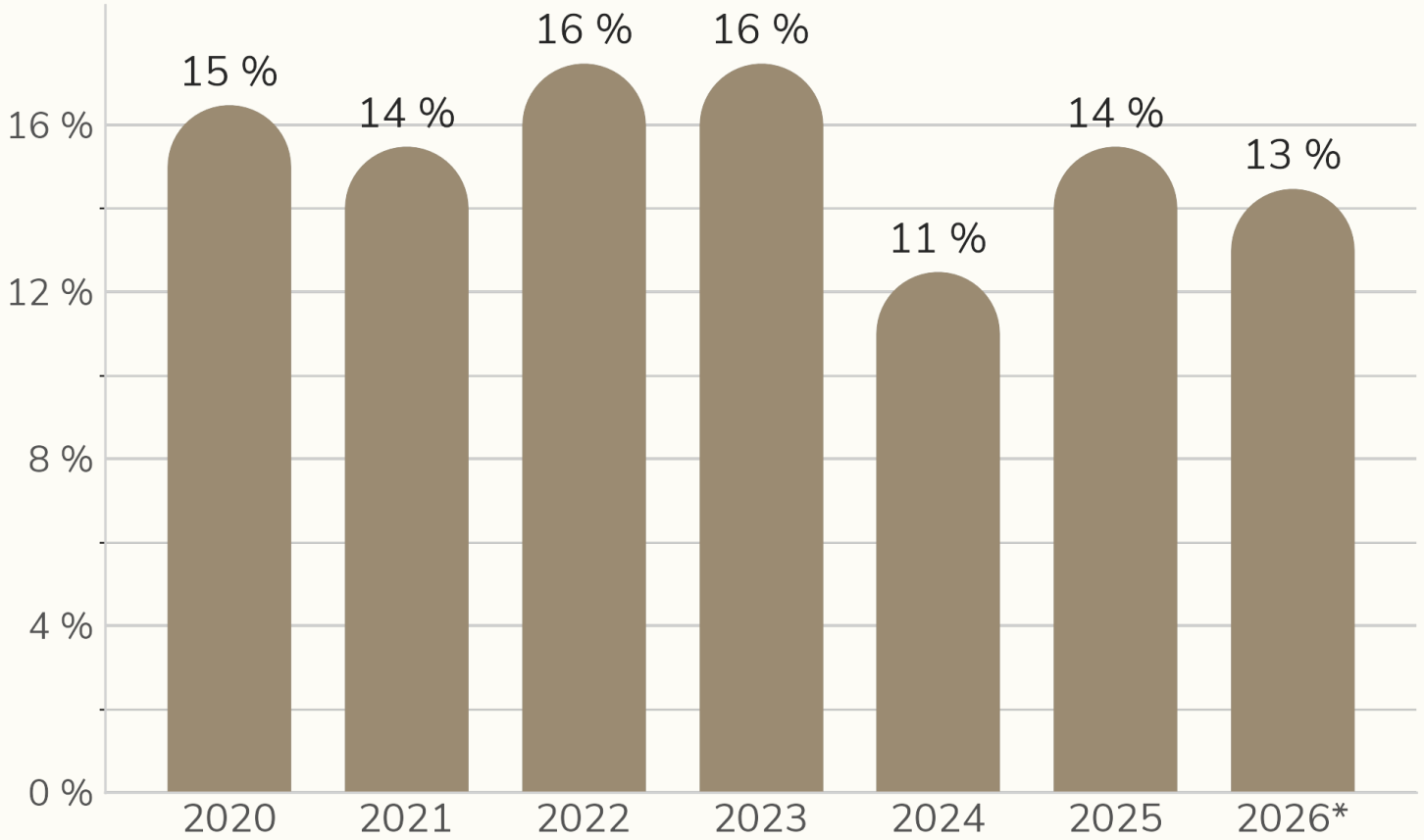
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Detailed information of the 22 OMPs with MA without NC (2/2)

	Brand name	Company	MA Date EMA	Waiting months (MA –30/04/2026)	Therapeutic area	ATMP
12	PYRUKYND	Agios	9/11/2022	42	Hematology	
13	RYTELO	Geron	7/03/2025	14	Oncology	
14	SEPHIENCE	PTC Therapeutics	19/06/2025	11	Metabolism	
15	SKYTROFA	Ascendis Pharma	11/01/2022	52	Hormone Preparations	
16	STRIMVELIS	Kyowa Kirin	26/05/2016	121	Immunomodulators	●
17	VORANIGO	Servier	17/09/2025	8	Oncology	
18	WASKYRA	Fondazione Telethon	09/01/2026	4	Hematology	●
19	XOLREMDI	X4 Pharmaceuticals	27/04/2026	0	Immunomodulators	
20	ZEMCELPRO	Cordex	25/08/2025	8	Hematology	●
21	ZIIHERA	Jazz Pharmaceuticals	27/06/2025	10	Oncology	
22	ZOKINVY	EigerBio	18/07/2022	46	Metabolism	

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Percentage of orphan medicinal products with MA without NC

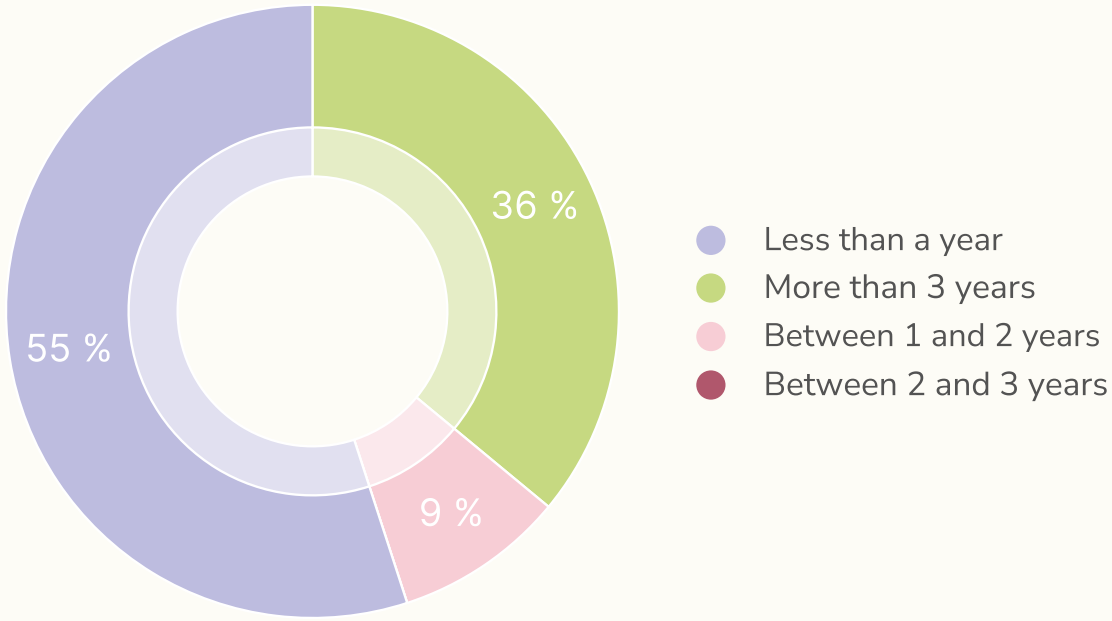


*Data accumulated until April 30, 2026.

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Orphan Medicinal Products with MA without NC by waiting years

By waiting years



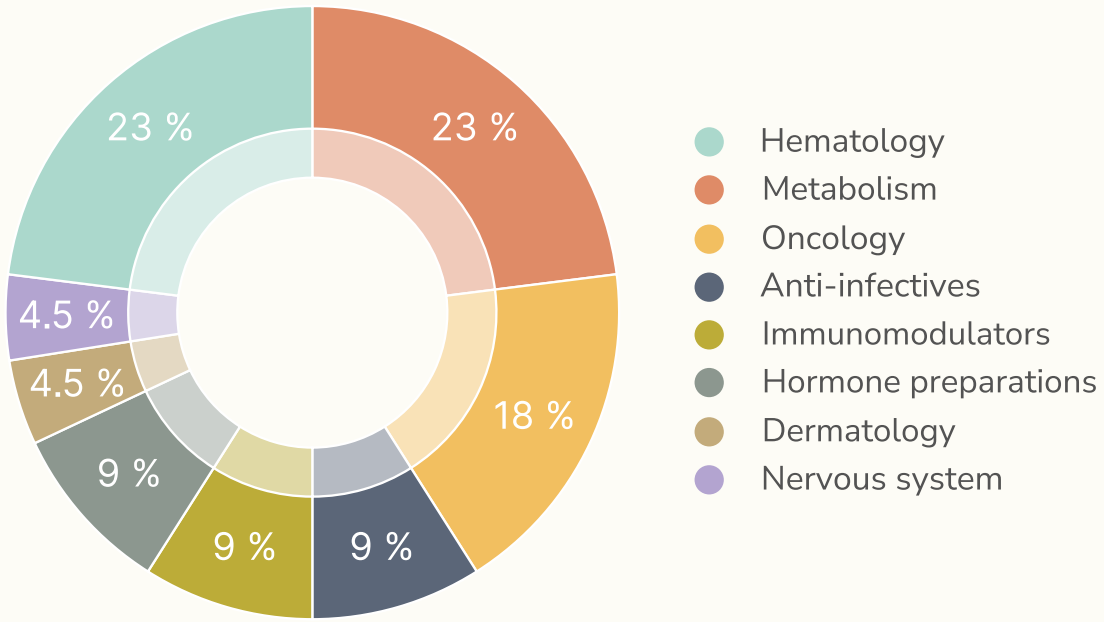
Absolute values



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Orphan Medicinal Products with MA without NC by therapeutic area

By therapeutic area



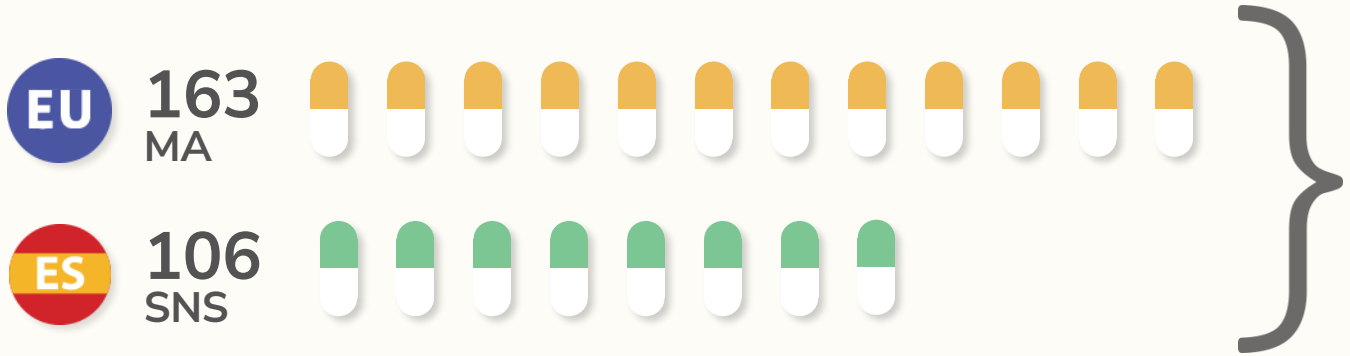
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Orphan
Medicinal
Products
Spain

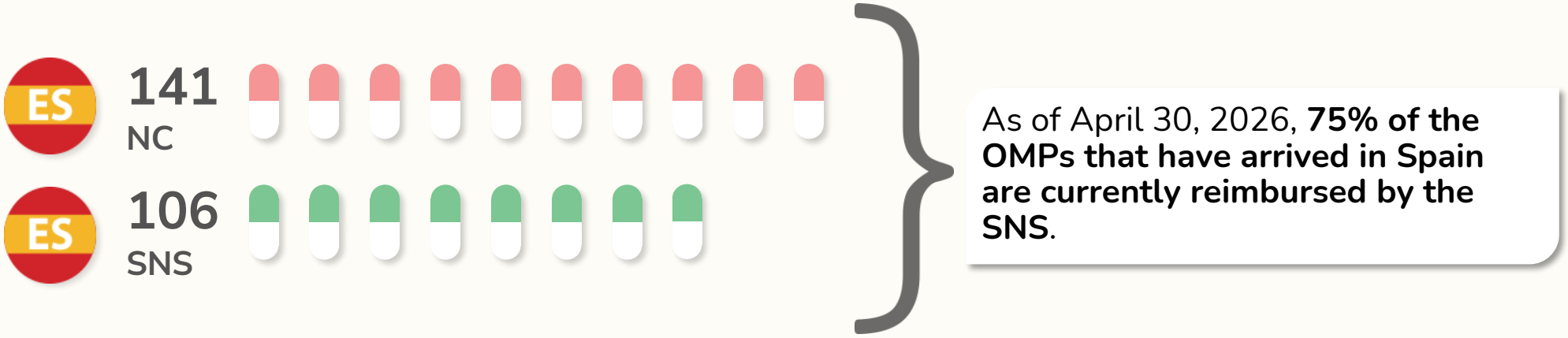
Orphan Medicinal Products with MA vs. reimbursed



As of April 30, 2026, **65% of the OMPs with MA are reimbursed by the SNS**.

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Comparison of OMPs with NC vs. reimbursed by the SNS

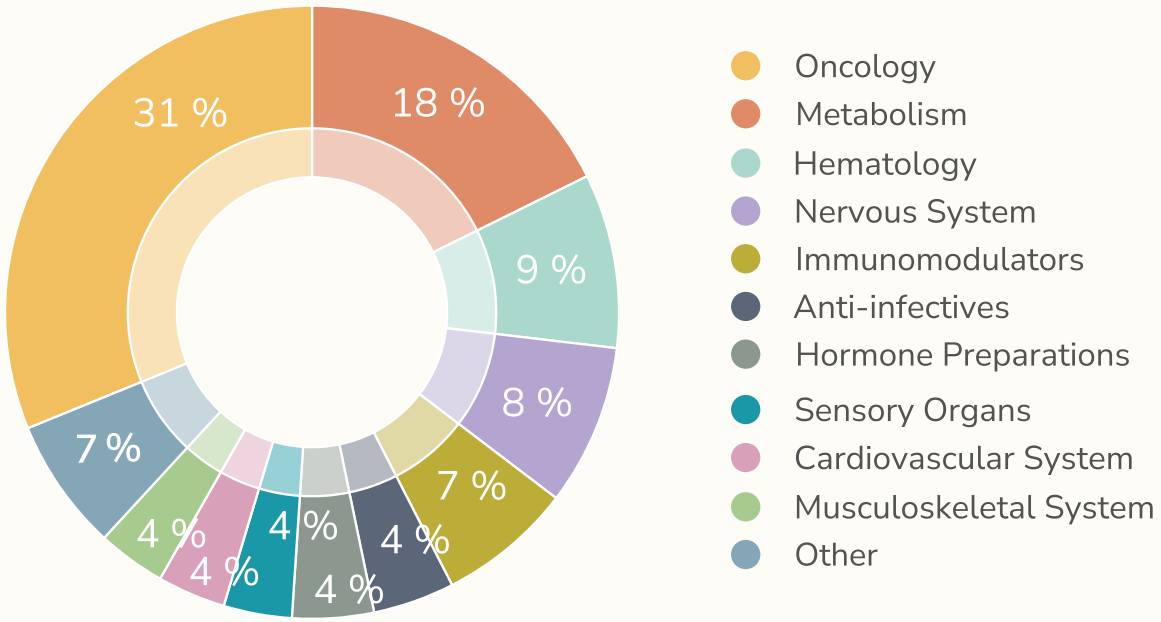


As of April 30, 2026, **75% of the OMPs that have arrived in Spain are currently reimbursed by the SNS**.

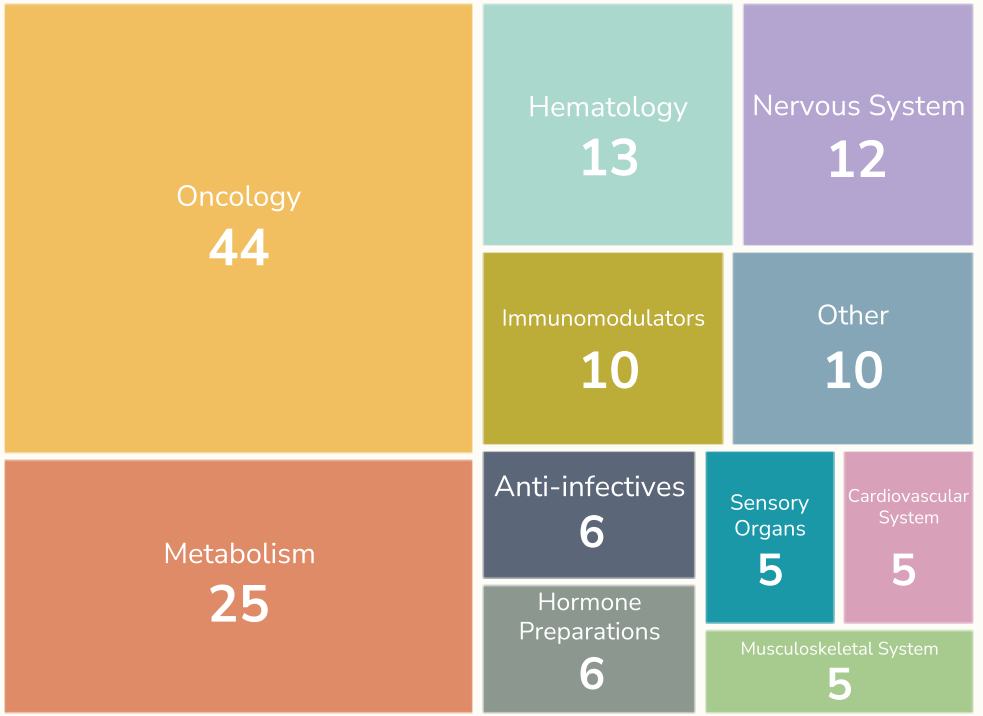
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OMPs with NC by therapeutic area

By therapeutic area



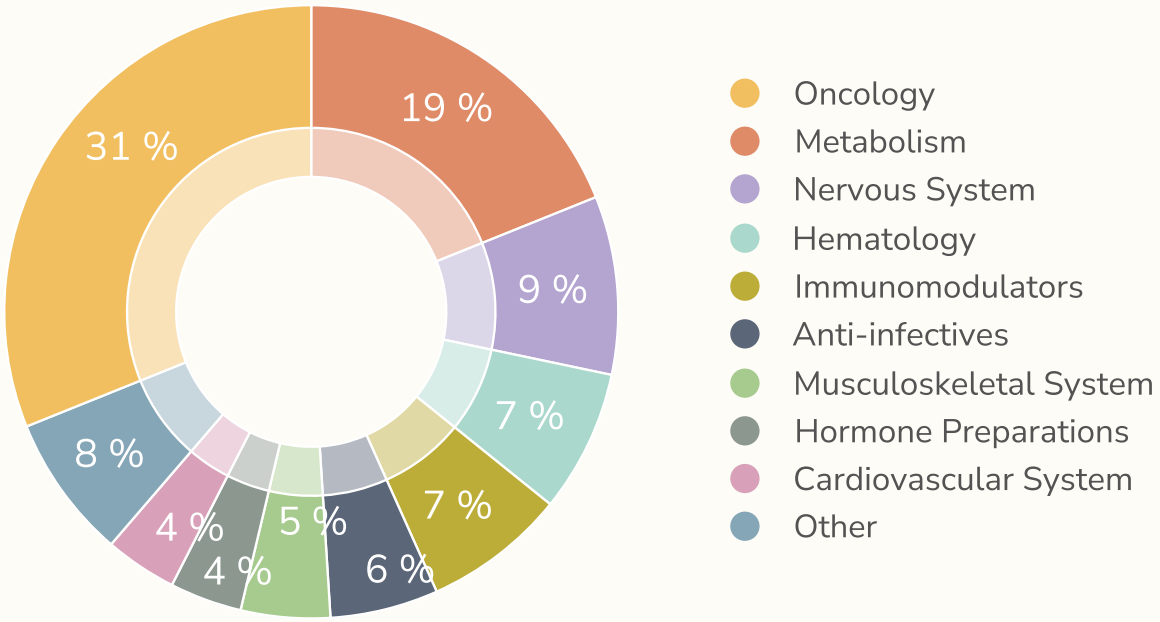
Absolute values



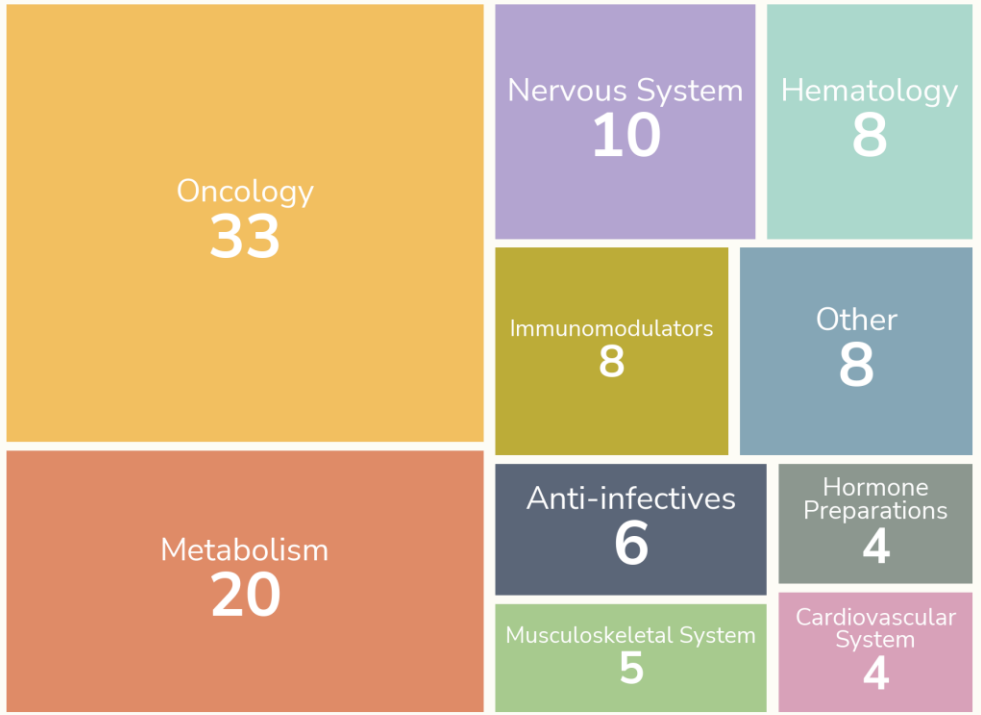
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OMPs reimbursed by therapeutic area

By therapeutic area

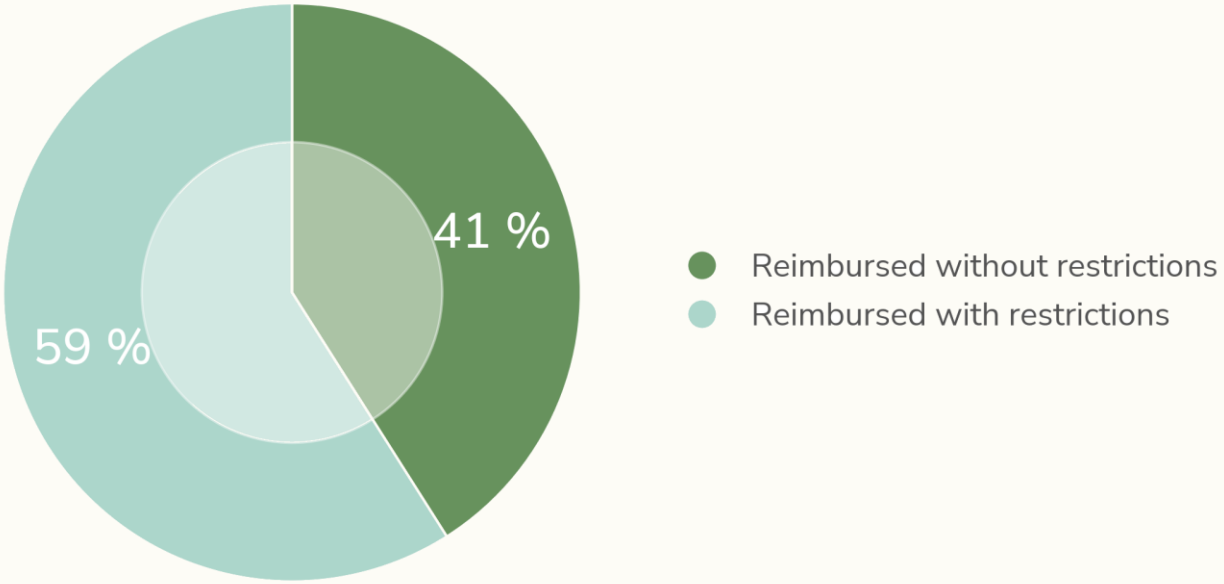


Absolute values



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Analysis of OMPs reimbursed by type of resolution

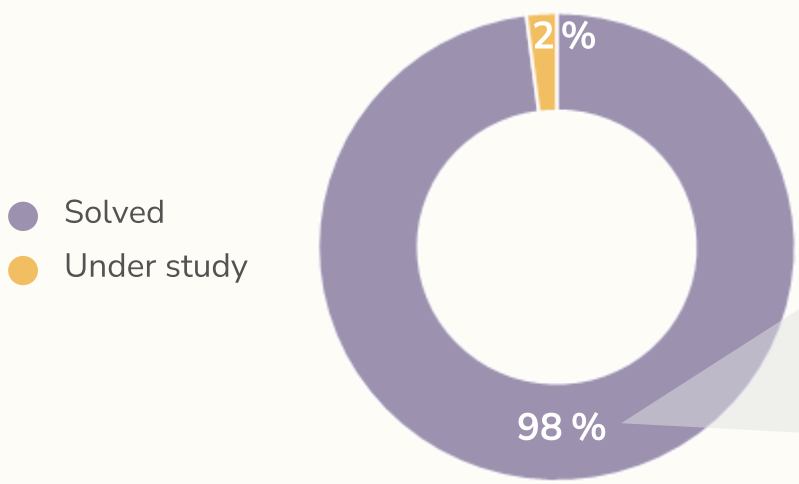


Of the 106 OMPs included in the SNS, **59% (63) are reimbursed with restrictions**, either due to restrictions on the authorized indication(s), or because they have some indication not reimbursed in our country.

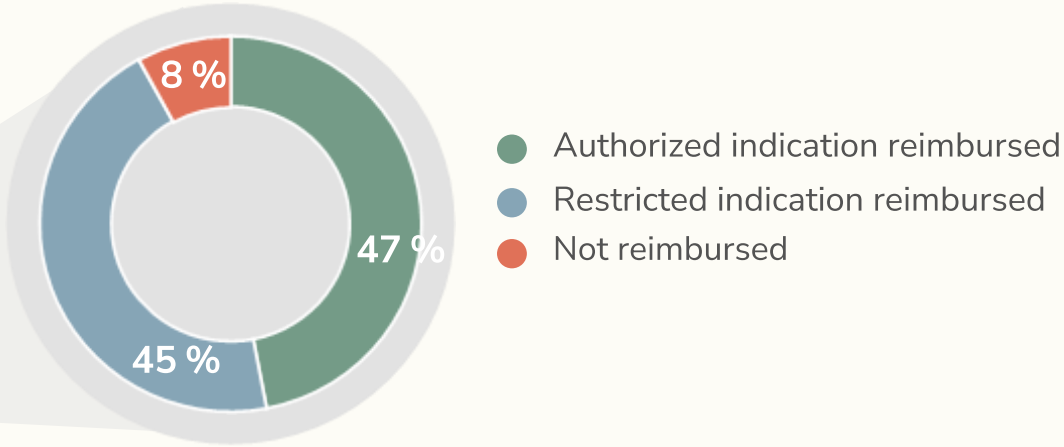
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Status of authorized indications of reimbursed OMPs

File status by indication



Resolution of the reimbursement file by indication

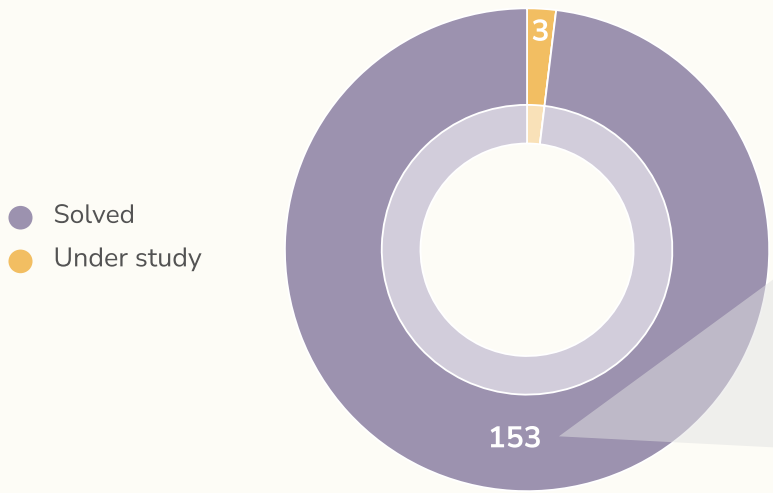


Source: BIFIMED (consulted in May 2026)

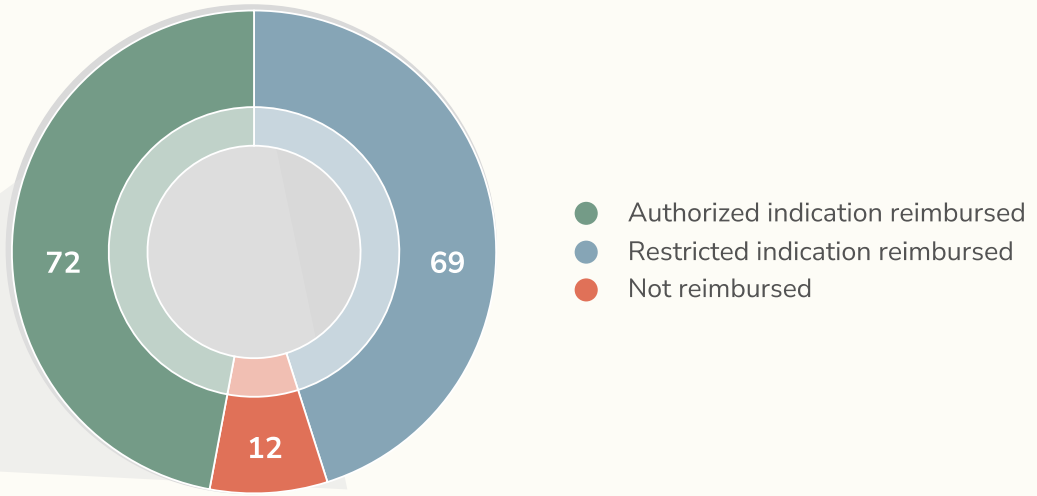
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Status of authorized indications of reimbursed OMPs

File status by indication
Absolute values



Resolution of the reimbursement file by indication
Absolute values



Of the 156 indications authorized by the EMA, 90% (141) are reimbursed.

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Orphan Medicinal Products
Reimbursed by the SNS between January and April of 2026

New orphan medicinal products reimbursed in 2026*

In the first four months of 2026, 3 new orphan medicinal products have been reimbursed in Spain.

4 months
Average time from MA to NC

5 months
Median time from MA to NC

22 months
Average time from NC to reimbursement

23 months
Median time from NC to reimbursement

100 %
Reimbursed with conditions by SNS

0 OMPs
reimbursed in the first year after obtaining NC

2 OMPs
have taken more than two years to be reimbursed after obtaining the NC

0 OMPs
reimbursed in their first CIPM meeting

2 OMPs
have attended at least four CIPM meetings before being reimbursed

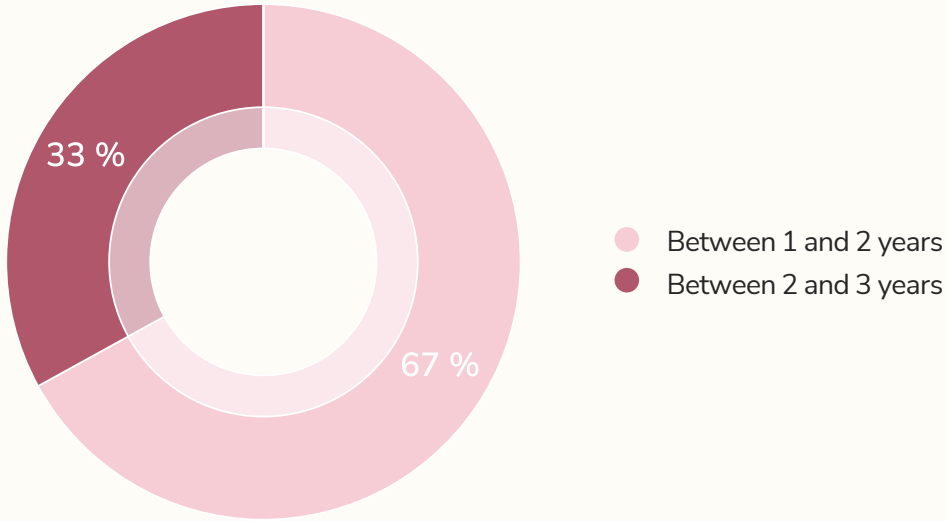
100 %
Reimbursed with restrictions

*Data accumulated until April 30, 2026.

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New orphan medicinal products reimbursed in 2026*

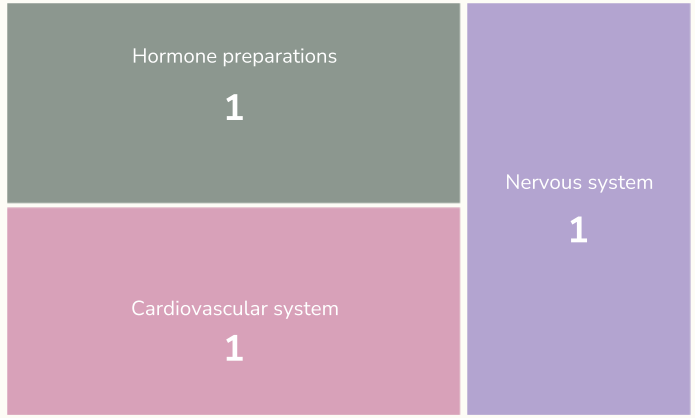
By waiting years



Absolute values by waiting years



Absolute values by therapeutic area



Note: The same medicine may be subject simultaneously to more than one reimbursement condition. For this reason, the sum of the different reimbursement conditions does not necessarily match the total of OMPs reimbursed during 2026.

*Data accumulated until April 30, 2026.

Source: BIFIMED (consulted in May 2026) and CIPM minutes (last published minutes: March 2026). Agreements of the Interministerial Commission on Drug Prices

In case of discrepancy between the information recorded in the CIPM minutes and BIFIMED, the information contained in the latter shall prevail.

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*New orphan medicinal products reimbursed in 2026**

OMPs reimbursed in 2026 by type of reimbursement condition

Reimbursement conditions	Number	%
Seguimed	3	100 %
Spending cap	2	67 %
Sales threshold	1	33 %
Refund of difference between Average Manufacturer Price (AMP) and price per Medicines under Special Situations (MSS)	2	67 %
Provision of a partial treatment or a free trial	1	33 %
Valtermed	0	0%

Note: The same medicine may be subject simultaneously to more than one reimbursement condition. For this reason, the sum of the different reimbursement conditions does not necessarily match the total of OMPs reimbursed during 2026.

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*Details of criteria and conditions of the 3 OMPs reimbursed by the SNS in 2026**

	Brand name	Company	NC Date	Reimbursement date	Decision on the reimbursement application	Months from MA to NC	Months from MA to reimbursement	CIPM Meetings	Reimbursement criteria[1]	Reimbursement conditions
1	**AGAMREE**	Santhera Pharmaceuticals	09/05/2024	01/04/2026	Yes, for certain indications	5	23	25/02/2026 28/01/2026 22/10/2025 19/06/2025	a) and c)	• Seguimed • Spending cap • Refund PVL/MSE • Provision of a partial treatment or a free trial
2	**FILSPARI**	Vifor	20/09/2024	01/04/2026	Yes, for certain indications	5	19	25/02/2026 17/12/2025	a) and c)	• Seguimed • Sales threshold
3	**SKYCLARYS**	Biogen	29/02/2024	01/03/2026	Yes, for certain indications	1	24	28/01/2026 19/11/2025 24/04/2025 29/01/2025	a) and c)	• Seguimed • Spending cap • Refund PVL/MSE

Source: BIFIMED (consulted in May 2026) and CIPM minutes (last published minutes: March 2026). Agreements of the Interministerial Commission on Drug Prices

In case of discrepancy between the information recorded in the CIPM minutes and BIFIMED, the information contained in the latter shall prevail.

*Data accumulated until April 30, 2026.

1 Criteria for reimbursement:

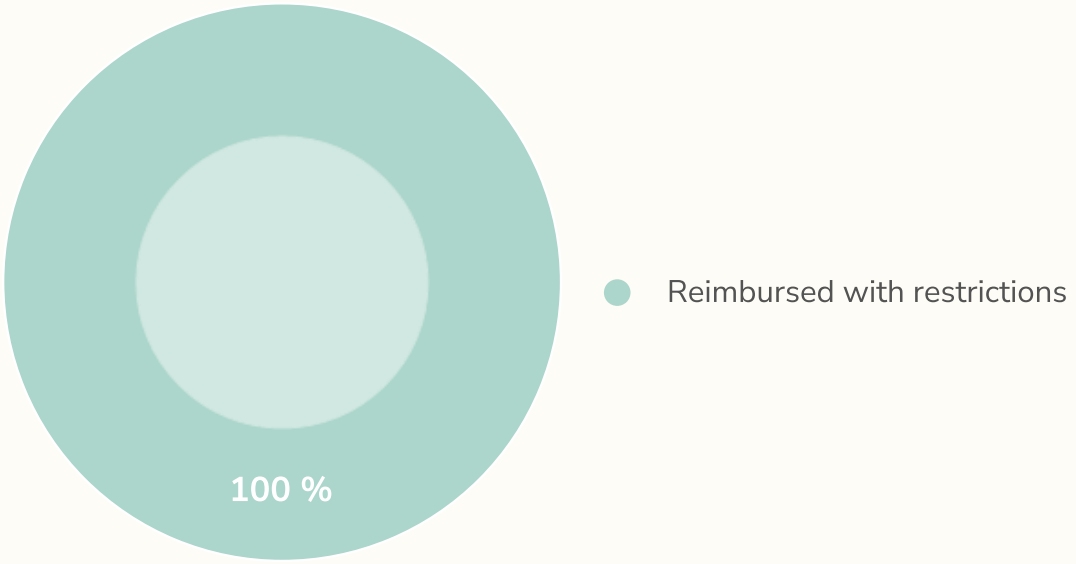
a) Severity, duration, and sequelae of the different pathologies for which they are indicated.

c) Therapeutic and social value of the medicine and its incremental clinical benefit considering its cost-effectiveness ratio.

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Analysis of the OMPs reimbursed in 2026 by type of resolution*

By type of resolution



Absolute values



Of the 3 orphan medicinal products reimbursed during the first four months of 2026, none are reimbursed without restrictions. All 3 are reimbursed with some type of restriction.

*Data accumulated until April 30, 2026.

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Detail of indications of the 3 OMPs reimbursed in 2026* (1/2)

	Brand name	Company	Reimbursement situation	Decision on the reimbursement application	Reimbursed indications (Ministry of Health)	EMA indication
1	AGAMREE	Santhera Pharmaceuticals	Yes, for certain indications	Yes, subject to the authorized indication	Coverage of AGAMREE® under the National Health System is limited to second-line treatment for patients aged 4 years or older with a diagnosis of Duchenne muscular dystrophy (DMD) confirmed by genetic testing, who have previously been treated with glucocorticoids and who require a dose reduction of more than 30% (deflazacort, average dose below 0.63 mg/kg/day) or require treatment discontinuation due to EA. Discontinuation Criteria: 1. If objective bone safety criteria are not met: if, after the first 24 months of treatment, a change of >3 points on the Spinal Deformity Index (SDI) occurs during any subsequent annual evaluation period, confirmed by reference to a lateral X-ray of the spine at the start of each 12-month evaluation period. Vertebral fractures caused by trauma will not be considered. 2. In case of unacceptable toxicity. 3. When the patient requires permanent mechanical ventilation.	AGAMREE is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 4 years of age and older.
2	FILSPARI	Vifor	Yes, for certain indications	Yes, subject to the authorized indication	The authorized indication is restricted to adults with biopsy-confirmed IgA nephropathy and a high risk of disease progression, with protein excretion ≥1.0 g/day (urine protein-to-creatinine ratio ≥ 0.75 g/g), who are being treated with stable doses of ACE inhibitors and/or ARBs at the maximum dose recommended in the package insert or at the maximum tolerated dose, and eGFR ≥30 mL/min/1.73 m². Not covered when used in combination with KINPEYGO®. Discontinuation criteria: - Patients who, after 36 weeks of treatment, have not achieved a ≥20% reduction in proteinuria relative to baseline, confirmed by at least two consecutive measurements under stable clinical conditions, and who maintain proteinuria in the high-risk range, urinary protein excretion ≥2.0 g/day, or a UPCR ≥1.76 g/g. - At any time after the initial evaluation at week 36 when, in re-evaluations conducted at least every 6 months, a ≥20% reduction in proteinuria from baseline is not maintained, confirmed by at least two consecutive measurements under stable clinical conditions, and proteinuria persists in the high-risk range. - Renal function (eGFR) will be monitored periodically as part of re-evaluations conducted at least every 6 months. The assessment of eGFR will be based on its evolution over time, using successive measurements taken under stable clinical conditions, after ruling out transient or potentially reversible causes. Confirmation of clinically relevant and sustained deterioration in renal function, as evidenced by changes in eGFR and defined as a decrease in eGFR of ≥30% from the baseline value prior to treatment, will be considered a criterion for reassessing clinical benefit and potential discontinuation. -The baseline proteinuria will be established at the start of treatment, once any prior non-concurrent treatment has been completed. -Measurements of proteinuria and renal function will be considered to have been performed under stable clinical conditions when the baseline treatment with ACE inhibitors and/or ARBs has not undergone significant changes in the preceding 4 weeks and the patient is free of recent acute intercurrent conditions.	Filspari is indicated for the treatment of adults with primary immunoglobulin A nephropathy (IgA nephropathy) with proteinuria of 1.0 g/day or greater (or a urine protein-to-creatinine ratio of 0.75 g/g or greater; see section 5.1).

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*Data accumulated until April 30, 2026.

Source: BIFIMED (consulted in May 2026)

Detail of indications of the 3 OMPs reimbursed in 2026* (2/2)

	Brand name	Company	Reimbursement situation	Decision on the reimbursement application	Reimbursed indications (Ministry of Health)	EMA indication
3	SKYCLARYS	Biogen	Yes, for certain indications	Yes, subject to the authorized indication	Criteria for initiating treatment: - Age ≥ 16 years - Genetically confirmed diagnosis of Friedreich's ataxia - Baseline mFARS < 80 - Patients of childbearing age who are not pregnant and are consistently using contraception - Controlled diabetes mellitus, HbA1c ≤ 11% - Normal liver function tests: ALT and AST ≤ 1.5 x ULN, alkaline phosphatase ≤ 2 x ULN, bilirubin ≤ 2 x ULN - No severe cardiomyopathy: Left ventricular ejection fraction ≥ 40% - No renal impairment: estimated glomerular filtration rate (eGFR ≥ 30 mL/min) - Life expectancy greater than 1 year due to Friedrich's ataxia. Clinical follow-up criteria: - Liver enzyme levels (AST, ALT) and bilirubin should be checked monthly for the first 3 months, then every 6 months - Every 6 months, complete blood count with differential, total cholesterol, LDL and HDL, eGFR, and BNP (or NT/proBNP) - Every 12 months, assess the patient's functional status using the specific Friedreich's ataxia mFARS scales. Discontinuation criteria: - Severely impaired functional status (mFARS ≥ 80 points) - Patients with uncontrolled diabetes mellitus, HbA1c ≥ 11% - Patients with severe liver failure, Child-Pugh class C - Patients with severe cardiomyopathy as determined by a specific evaluation by a cardiologist, NYHA Class IV - Patients with renal impairment, estimated glomerular filtration rate (eGFR) < 30 mL/min - Women who are pregnant or planning to become pregnant, who must discontinue treatment at least 28 days in advance - Patients with elevated liver enzymes (ALT or AST) > 5 times the upper limit of normal (ULN) and bilirubin > 2 times the ULN, and who do not return to normal enzyme levels after a 4-week treatment break - An increase of > 2 points on the mFARS scale at 12 months.	Skyclarys is indicated for the treatment of Friedreich's ataxia in adults and adolescents 16 years of age and older.

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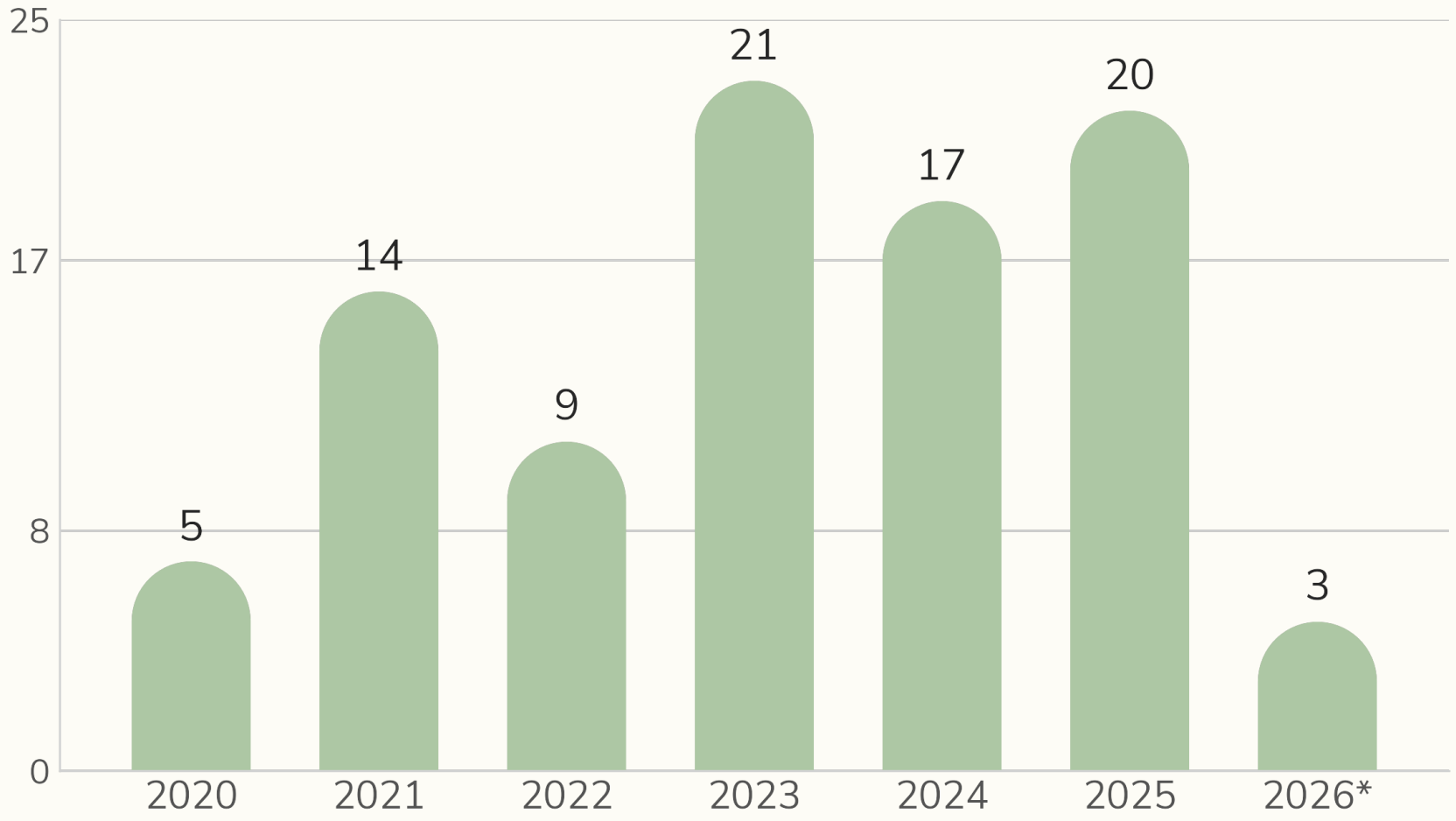
*Data accumulated until April 30, 2026.

Source: BIFIMED (consulted in May 2026)

*Number of reimbursed OMPs in Spain 2020-2026**

ES

SNS

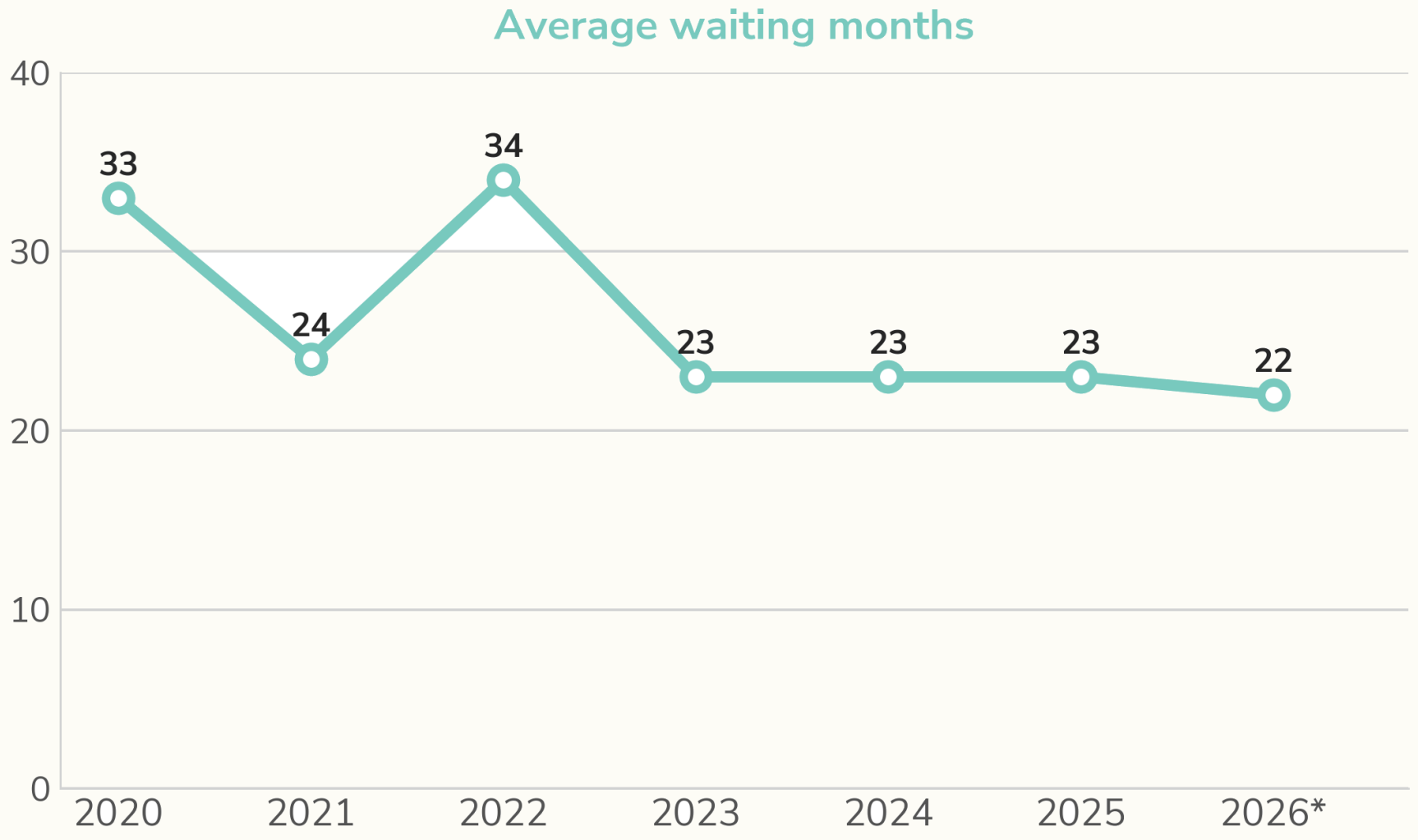


Note: The chart shows the annual situation of reimbursed medicines, however, the data are not cumulative year over year.

*Data accumulated until April 30, 2026.

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Average waiting time from NC to reimbursement (2020–2026*)



*Data accumulated until April 30, 2026.

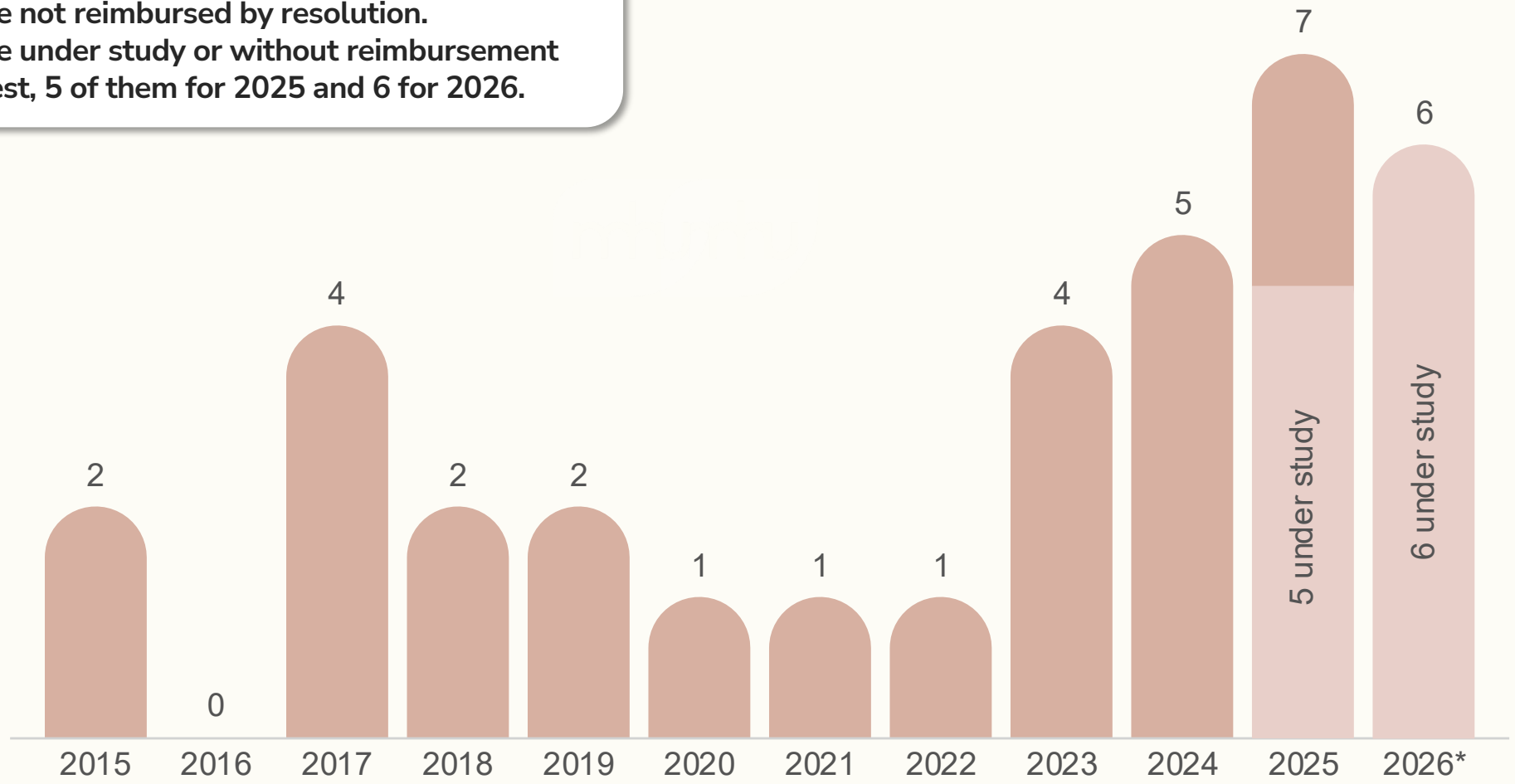
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Orphan
Medicinal
Products
not reimbursed

Orphan Medicinal Products not reimbursed in Spain by NC year

35 orphan medicinal products not reimbursed:

- **24 are not reimbursed by resolution.**
- **11 are under study or without reimbursement request, 5 of them for 2025 and 6 for 2026.**

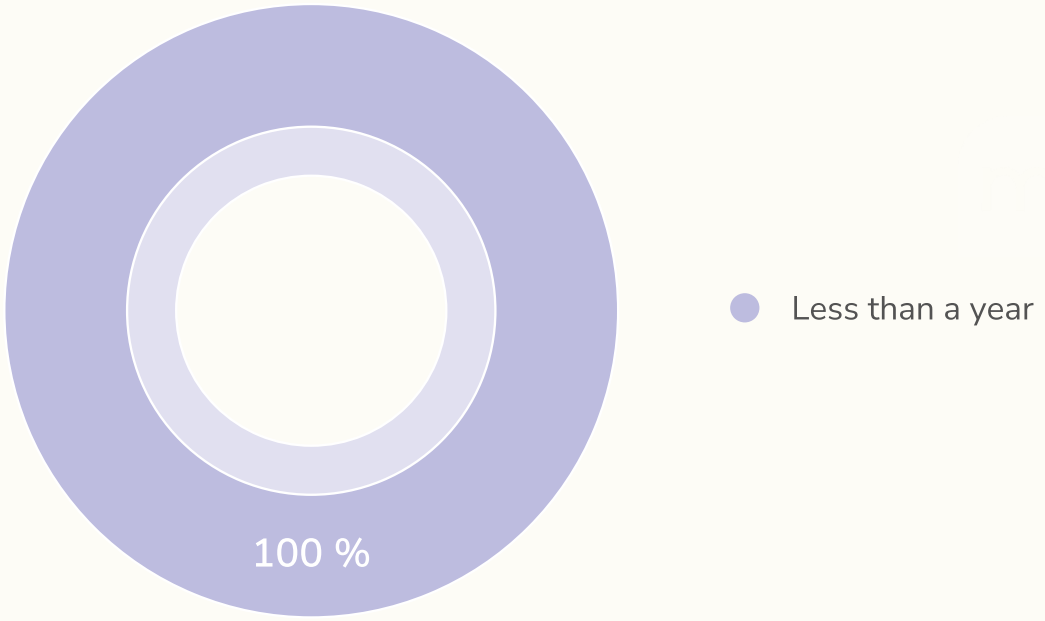


*Data accumulated until April 30, 2026.

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Orphan Medicinal Products under reimbursement study with NC

Years of waiting for reimbursement



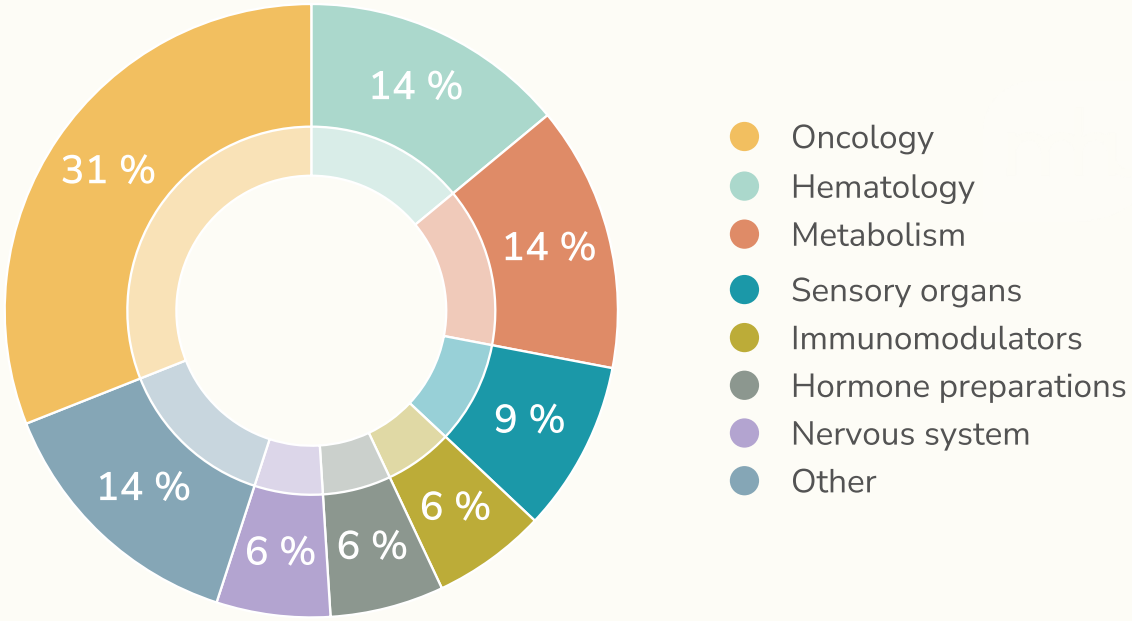
Absolute values



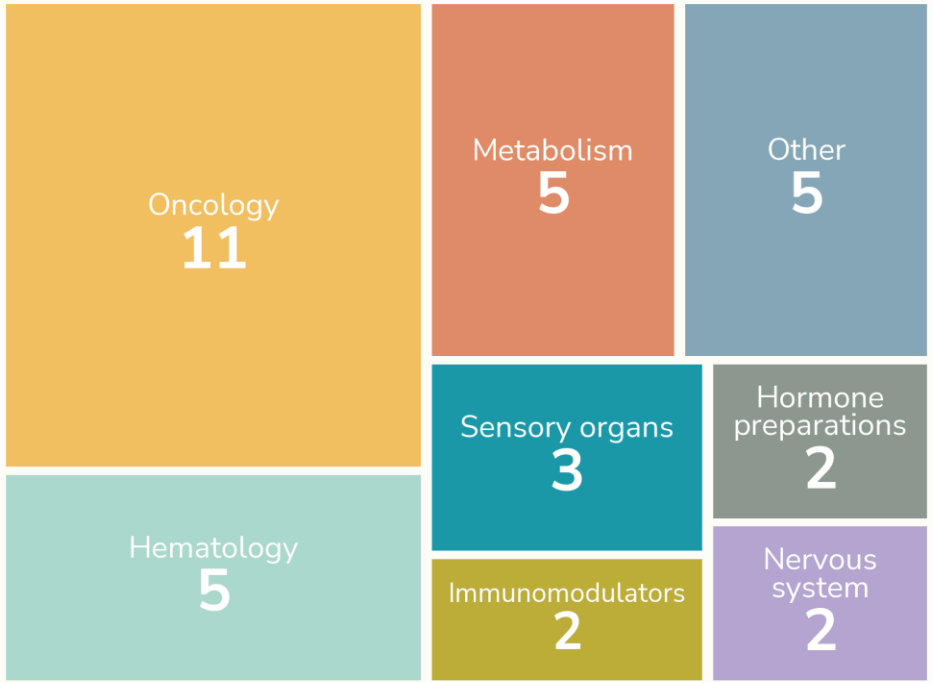
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OMPs with NC pending reimbursement

Classification by therapeutic area



Absolute values



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OMPs with NC pending reimbursement since 2026*

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement/ exclusion	Criteria for non-reimbursement[1]	Months since NC
1	**EZMEKLY**	Oncology	SpringWorks Therapeutics	18/02/2026	-	-	-	2
2	**OGSIVEO**	Oncology	SpringWorks Therapeutics	02/01/2026	-	-	-	4
3	**REZUROCK**	Oncology	Sanofi	31/03/2026	-	-	-	1
4	**VORANIGO**	Oncology	Servier	02/01/2026	-	-	-	4
5	**WAYRILZ**	Hematology	Sanofi	17/04/2026	-	-	-	0
6	**ZYNYZ**	Oncology	Incyte	02/03/2026	-	-	-	2

*Data accumulated until April 30, 2026.

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OMPs with NC pending reimbursement since 2025

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement /exclusion*	Criteria for non-reimbursement[1]	Months since NC
1	**ALYFTREK**	Respiratory system	Vertex Pharmaceuticals	13/08/2025	25/02/2026	-	d)	9
2	**ARTESUNATE**	Antiparasitic	Amivas	14/01/2025	24/09/2025 16/07/2025	01/11/2025	d) and e) d) and e)	16
3	**COAGADEX**	Hematology	BPL Bioproducts	31/03/2025	-	01/03/2025	-	13
4	**ENJAYMO**	Immunomodulators	Sanofi	29/12/2025	-	-	-	4
5	**ROMVIMZA**	Oncology	Deciphera Pharmaceuticals	26/11/2025	-	-	-	5
6	**TRYNGOLZA**	Cardiovascular system	SOBI	06/11/2025	-	-	-	6
7	**VYJUVEK****	Dermatology	Krystal Biotech	24/10/2025	-	-	-	6

* The non-reimbursement date indicated in BIFIMED is the first date of non-reimbursement/exclusion

**ATMP with orphan designation

1 Criteria for non-reimbursement:

d) Rationalization of public expenditure allocated to pharmaceutical provision and budgetary impact on the National Health System.
e) Existence of medicines or other therapeutic alternatives for the same conditions at a lower price or lower treatment cost.

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OMPs with NC pending reimbursement since 2024

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement/ exclusion*	Criteria for non-reimbursement[1]	Months since NC
1	**ADZYNMA**	Hematology	Takeda	30/10/2024	28/01/2026 19/11/2025	01/03/2026	d)	18
2	**CASGEVY**	Hematology	Vertex Pharmaceuticals	01/03/2024	19/11/2025 19/06/2025	01/01/2026	d) d)	26
3	**ELAHERE****	Oncology	Abbvie	04/12/2024	-	01/03/2026	-	17
4	**VYLOY**	Oncology	Astellas	30/09/2024	24/09/2025	01/03/2026	d) and e)	19
5	**ZTALMY****	Nervous system	Marinus Pharmaceuticals	18/06/2024	-	01/10/2025	-	23

* The non-reimbursement date indicated in BIFIMED is the first date of non-reimbursement/exclusion

**ATMP with orphan designation

** ELAHERE and ZTALMY have not attended any CIPM meeting. Although they already have an official non-reimbursement date, there are no public sources that allow knowing the reason for the decision.

1 Criteria for non-reimbursement:

d) Rationalization of public expenditure allocated to pharmaceutical provision and budgetary impact on the National Health System.
e) Existence of medicines or other therapeutic alternatives for the same conditions at a lower price or lower treatment cost.

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OMPs with NC pending reimbursement since 2023

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement/ exclusion*	Criteria for non-reimbursement[1]	Months since NC
1	**EBVALLO****	Other	Pierre	12/01/2023	25/03/2026 19/11/2025 20/02/2025 20/11/2024	01/04/2025	d) c) and d) c) and d) c) and d)	40
2	**ELZONRIS**	Oncology	Stemline Therapeutics	20/10/2023	29/01/2025 17/07/2024	01/03/2025	c) and d)	31
3	**ROCTAVIAN****	Hematology	BioMarin	03/02/2023	19/06/2024	01/10/2024	c) and d)	39
4	**SOGROYA**	Hormone Preparations	Novo Nordisk	08/11/2023	24/04/2024	01/07/2024	d) and e)	30

* The non-reimbursement date indicated in BIFIMED is the first date of non-reimbursement/exclusion

**ATMP with orphan designation

1 Criteria for non-reimbursement:

c) Therapeutic and social value of the medicine and its incremental clinical benefit taking into account its cost-effectiveness ratio.
d) Rationalization of public expenditure allocated to pharmaceutical provision and budgetary impact on the National Health System.
e) Existence of medicines or other therapeutic alternatives for the same conditions at a lower price or lower treatment cost.

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OMPs with NC pending reimbursement since 2022

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement/ exclusion*	Criteria for non-reimbursement[1]	Months since NC
1	**UPSTAZA****	Nervous system	PTC Therapeutics	22/12/2022	23/11/2023 11/05/2023	01/01/2024	d) d)	41

* The non-reimbursement date indicated in BIFIMED is the first non-reimbursement/exclusion date

**ATMP with orphan designation

1 Criteria for non-reimbursement:

d) Rationalization of public spending allocated to pharmaceutical provision and budgetary impact on the National Health System.

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OMPs with NC pending reimbursement since 2021

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement /exclusion*	Criteria for non-reimbursement[1]	Months since NC
1	**AMGLIDIA**	Metabolism	Ammtek	14/01/2021	30/09/2021 08/07/2021	01/12/2021	d) c) and d)	64

* The non-reimbursement date indicated in BIFIMED is the first non-reimbursement/exclusion date

1 Criteria for non-reimbursement:

c) Therapeutic and social value of the medicine and its incremental clinical benefit considering its cost-effectiveness ratio.
d) Rationalization of public expenditure allocated to pharmaceutical provision and budgetary impact on the National Health System.

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OMPs with NC pending reimbursement since 2020

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement/ exclusion*	Criteria for non-reimbursement[1]	Months since NC
1	**DAURISMO**	Oncology	Pfizer	24/07/2020	04/04/2022 16/12/2021	01/06/2022	d) and e) c) and d)	70

* The non-reimbursement date indicated in BIFIMED is the first date of non-reimbursement/exclusion

1 Criteria for non-reimbursement:

c) Therapeutic and social value of the medicine and its incremental clinical benefit taking into account its cost-effectiveness ratio.
d) Rationalization of public expenditure allocated to pharmaceutical provision and budgetary impact on the National Health System.
e) Existence of medicines or other therapeutic alternatives for the same conditions at a lower price or lower treatment cost.

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OMPs with NC pending reimbursement since 2019

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement/ exclusion*	Criteria for non-reimbursement[1]	Months since NC
1	**PALYNZIQ**	Metabolism	BioMarin	16/12/2019	02/06/2022 03/03/2022 10/06/2021	01/10/2021	c) and d) c) c)	78
2	**VERKAZIA**	Immunomodulators	Santen Pharmaceutical	08/04/2019	07/04/2021	01/07/2021	d) and e)	86

* The non-reimbursement date indicated in BIFIMED is the first date of non-reimbursement/exclusion

1 Criteria for non-reimbursement:

c) Therapeutic and social value of the medicine and its incremental clinical benefit taking into account its cost-effectiveness ratio.
d) Rationalization of public expenditure allocated to pharmaceutical provision and budgetary impact on the National Health System.
e) Existence of medicines or other therapeutic alternatives for the same conditions at a lower price or lower treatment cost.

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OMPs with NC pending reimbursement since 2018

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement/ exclusion*	Criteria for non-reimbursement[1]	Months since NC
1	**MYALEPTA**	Metabolism	Chiesi	06/11/2018	25/11/2021 10/06/2021 17/06/2020 28/11/2019	01/08/2020	c) and d) c) and d) d) c) and d)	91
2	**NATPAR**	Hormone Preparations	Takeda	12/02/2018	-	01/02/2019	-	100

* The non-reimbursement date indicated in BIFIMED is the first date of non-reimbursement/exclusion

1 Criteria for non-reimbursement:

c) Therapeutic and social value of the medicine and its incremental clinical benefit taking into account its cost-effectiveness ratio.
d) Rationalization of public expenditure allocated to pharmaceutical provision and budgetary impact on the National Health System.

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OMPs with NC pending reimbursement since 2017

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement/ exclusion*	Criteria for non-reimbursement[1]	Months since NC
1	**CYSTADROPS**	Sense organs	Recordati	27/12/2017	05/05/2022 03/03/2022	01/12/2018	e) and d) e) and d)	102
2	**NINLARO**	Oncology	Takeda	06/02/2017	07/04/2021 03/02/2021	01/03/2019	d) d)	112
3	**OXERVATE**	Sense organs	Dompe	02/10/2017	-	01/07/2019	-	104
4	**XERMELO**	Metabolism	SERB	22/12/2017	-	01/06/2018	-	102

* The non-reimbursement date indicated in BIFIMED is the first date of non-reimbursement/exclusion

1 Criteria for non-reimbursement:

d) Rationalization of public expenditure allocated to pharmaceutical provision and budgetary impact on the National Health System.
e) Existence of medicines or other therapeutic alternatives for the same conditions at a lower price or lower treatment cost.

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OMPs with NC pending reimbursement since 2015

	Brand name	Therapeutic area	Company	NC Date	CIPM Meetings	Date of non-reimbursement/exclusion*	Criteria for non-reimbursement[1]	Months since NC
1	**HOLOCLAR**	Sense organs	Holostem	19/09/2015	-	01/08/2016	-	129
2	**STRENSIQ**	Metabolism	Alexion	15/10/2015	22/05/2024 20/03/2024	01/07/2018	d) d)	128

* The non-reimbursement date indicated in BIFIMED is the first date of non-reimbursement/exclusion

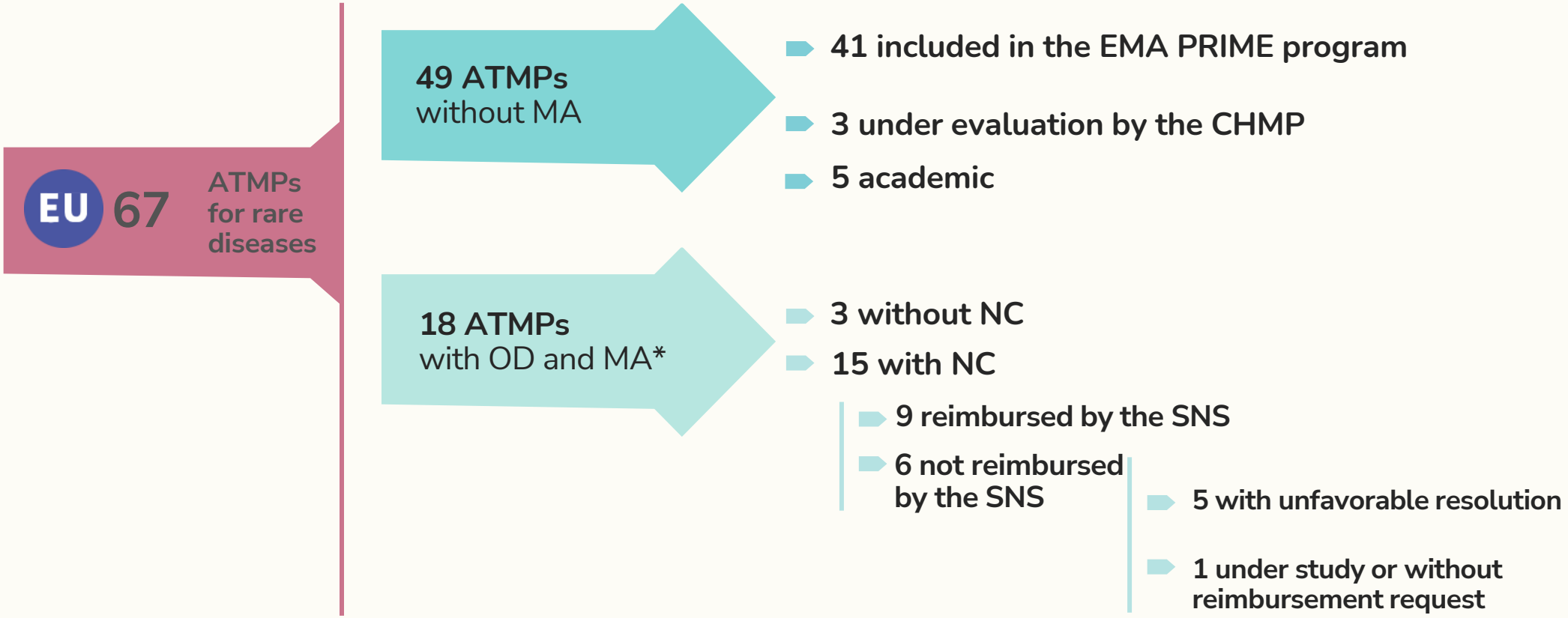
1 Criteria for non-reimbursement:

d) Rationalization of public spending allocated to pharmaceutical provision and budgetary impact on the National Health System.

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Advanced Therapy Medicinal Products (ATMPs)
for rare diseases

Advanced Therapy Medicinal Products for rare diseases



*The 18 advanced therapy medicinal products with OD and MA are included in the total of 230 OMPs with OD and in the 163 OMPs with MA, since they are ATMPs with a brand name.

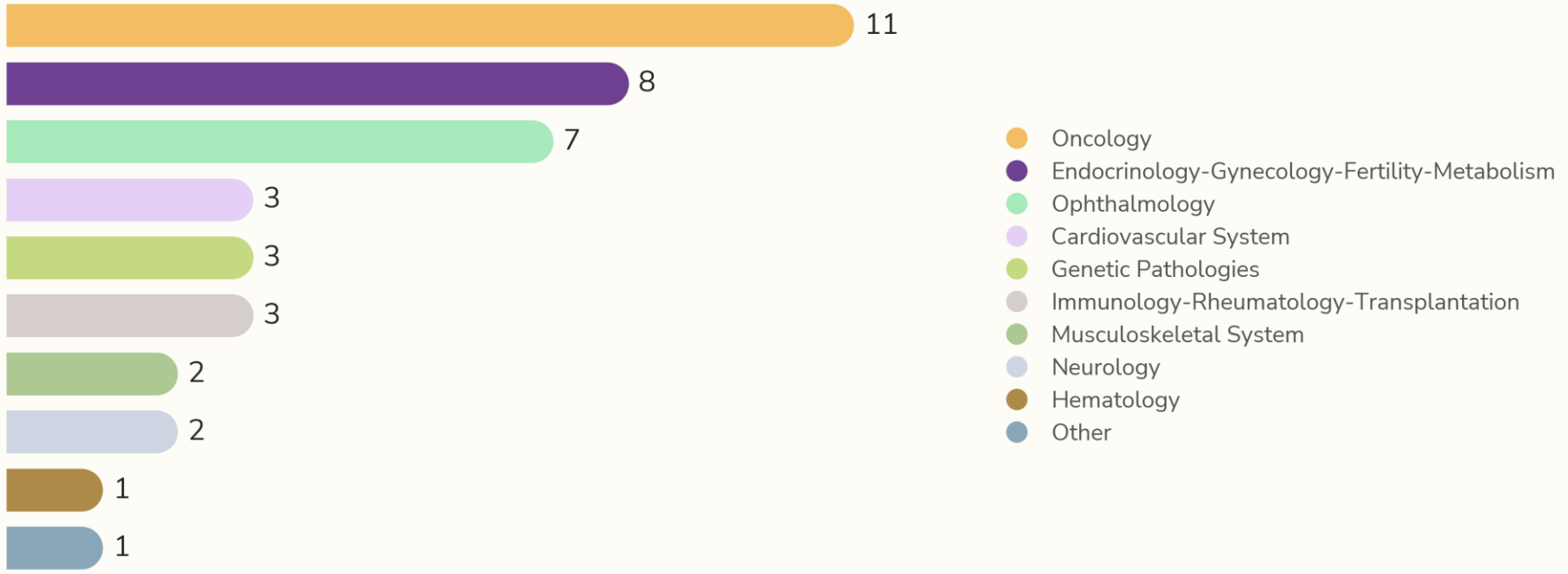
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ATMPs for rare diseases included in the EMA PRIME program

41

EU

Classification by therapeutic area



The PRIME program is intended to support the development of medicines that address an unmet medical need. This voluntary scheme is based on increased interaction and early dialogue with innovative medicine companies so that these therapies can reach patients sooner.

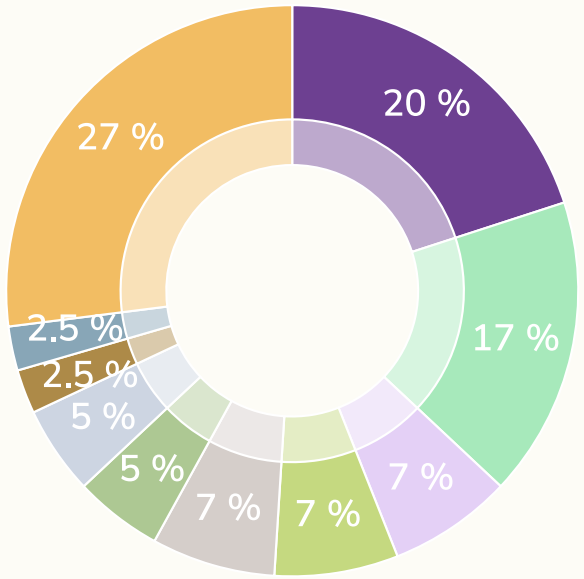
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ATMPs for rare diseases included in the EMA PRIME program

41

EU

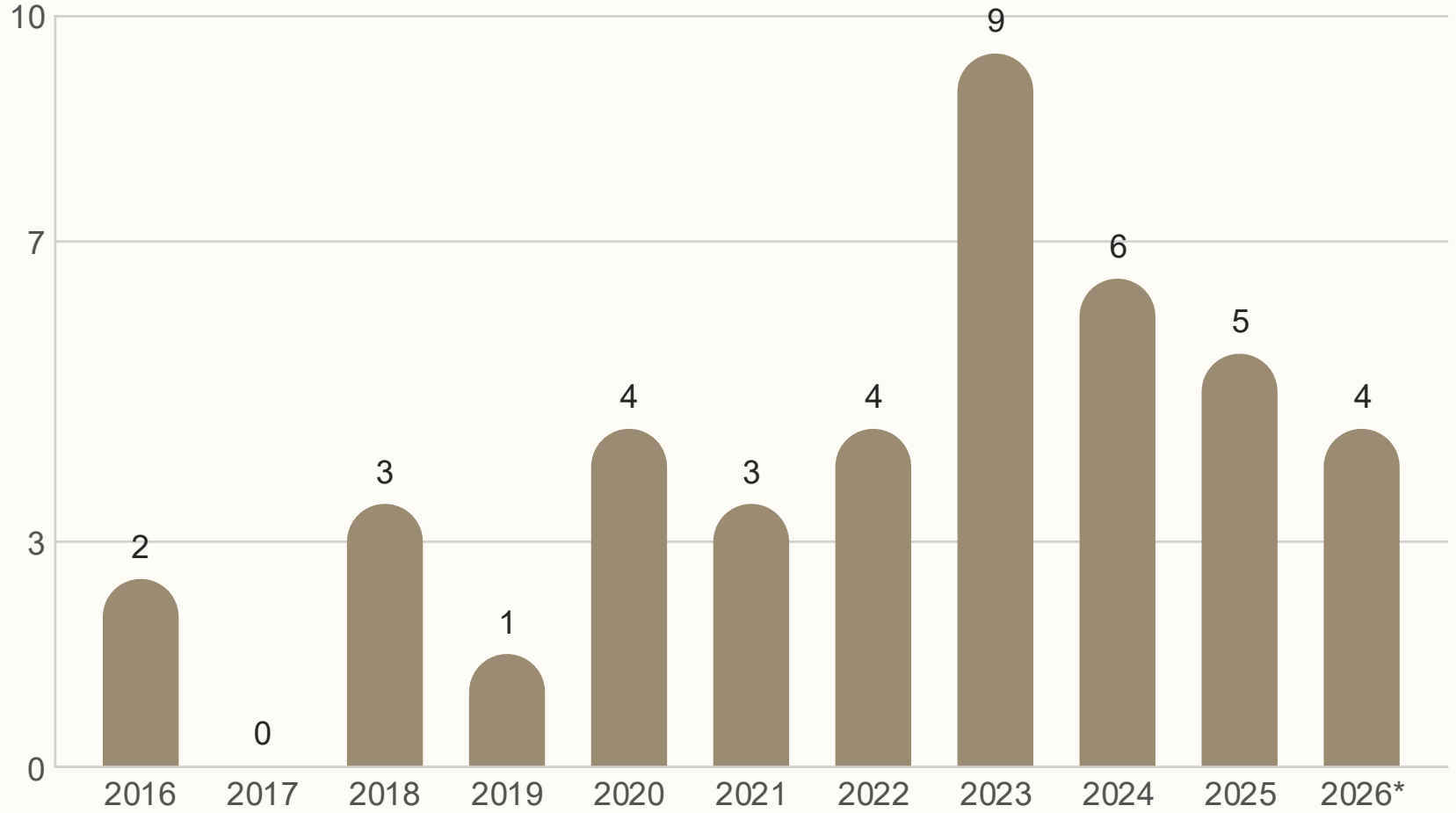
Classification by therapeutic area



- Oncology
- Endocrinology-Gynecology-Fertility-Metabolism
- Ophthalmology
- Cardiovascular System
- Genetic Pathologies
- Immunology-Rheumatology-Transplantation
- Musculoskeletal System
- Neurology
- Hematology
- Other

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ATMPs for rare diseases included in the EMA PRIME program (2016-2026)*



The chart indicates the year each ATMP was incorporated into the PRIME program.

*Data accumulated until April 30, 2026.

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Details of ATMPs for rare diseases under evaluation by the CHMP

	Name**	Company	Therapeutic area	Therapeutic indication	Evaluation start date
1	**Onasemnogene abeparvovec***	Novartis	Musculoskeletal system	Treatment of 5q spinal muscular atrophy	22/05/2025
2	**Zamtocabtagene autoleucel**	Miltenyi Biomedicine	Oncology	Treatment of mature B-cell neoplasms	26/03/2026
3	**Zopapogene imadenovec**	FGK Representative Services GmbH	Respiratory system	Treatment of respiratory papillomatosis in adults	27/11/2025

*This ATMP has been authorized by the EMA since 2020, but reappears under evaluation at the CHMP as of 05/22/2025.

**International Nonproprietary Name (INN)/ common name.

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Details of academic ATMPs for rare diseases

	Name	Therapeutic area	Authorization holder	Date of approval
1	**ARI-0001 Infusion suspension containing 0.1–1 × 10^6 cells/kg – Hospital Clínic de Barcelona**	Oncology	Hospital Clínic de Barcelona	01/02/2021
2	**ARI0002H 13.5–720 × 10^6 cells for perfusion**	Oncology	Hospital Clínic de Barcelona	08/08/2024
3	**CEMTROCELL 50,000 cells/microliter – Suspension for implantation**	Musculoskeletal system	Clínica Cemtro S.A.	31/10/2023
4	**NC1—Cell suspension in autologous plasma, 100–300 × 10^6 cells – Puerta de Hierro Majadahonda University Hospital, 1 pre-filled syringe**	Neurology	Puerta de Hierro Majadahonda University Hospital	31/01/2019
5	**Tissue-engineered human skin 1.0–1.5 × 10^6 / 1.5–3.0 × 10^6 cells per impregnated dressing sheet**	Dermatology	Virgen del Rocío University Hospital – Seville	06/06/2024

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Approval details of the 18 ATMPs with OD and MA

18 ATMPs

Type of approval of ATMPs with OD and MA*



- Standard Approval
- Approved Under Exceptional Circumstances
- Conditional Approval

Special conditions of approval of ATMPs with OD and MA*



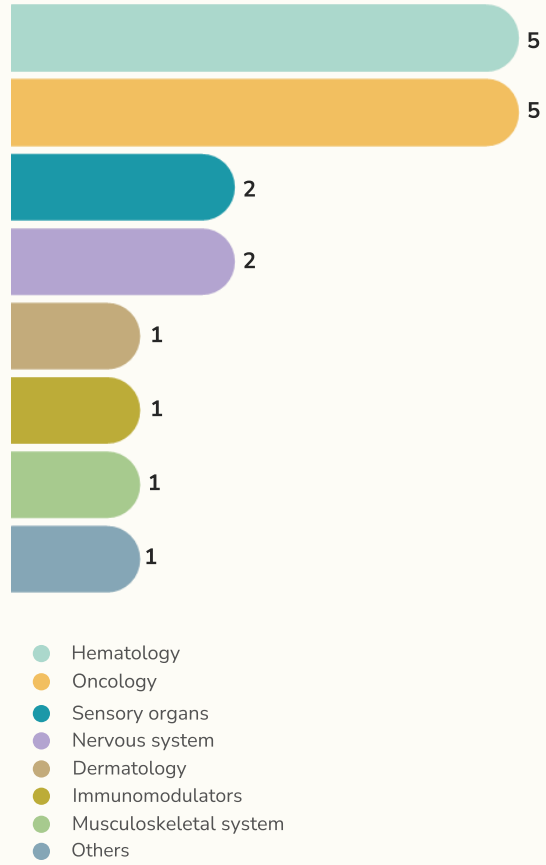
- Additional Follow-up

*The 18 advanced therapy medicinal products with OD and MA are included in the total of 230 OMPs with OD and in the 163 OMPs with MA, since they are ATMPs with a commercial name.

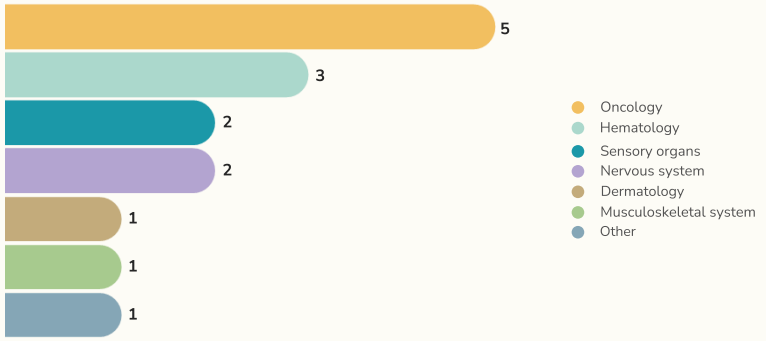
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Classification of the 18 ATMPs with OD and MA by therapeutic area

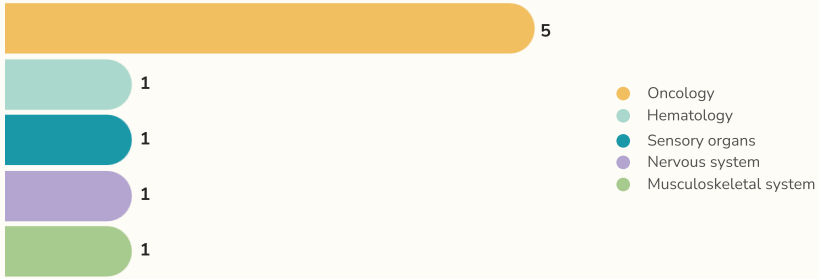
ATMPs with OD and MA by therapeutic area



ATMPs with OD and NC by therapeutic area



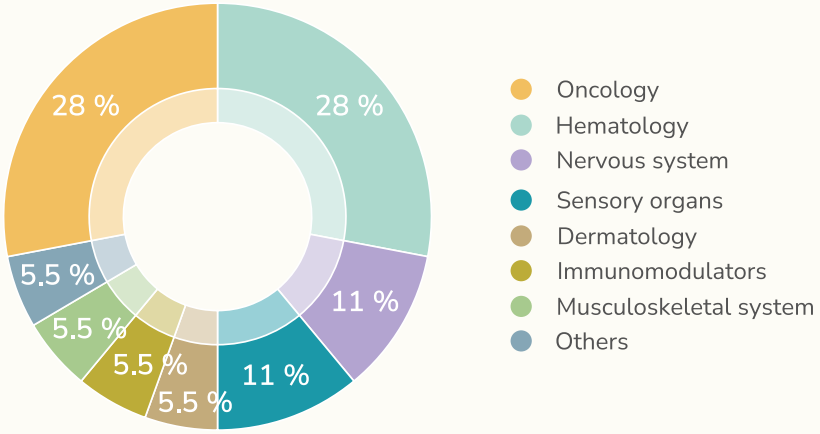
ATMPs with OD and reimbursed in Spain by therapeutic area



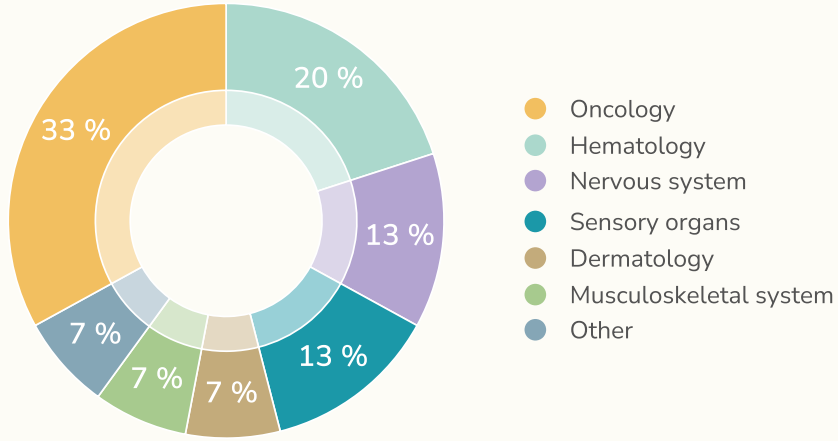
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Classification of the 18 ATMPs with OD and MA by therapeutic area

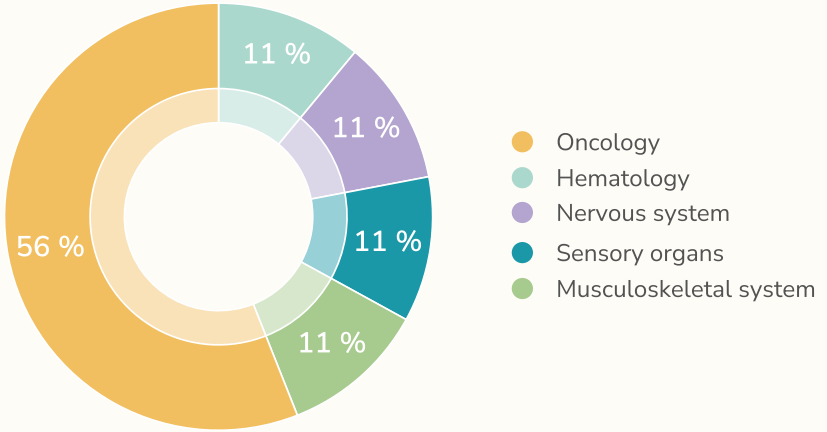
EU ATMPs with OD and MA by therapeutic area



ES ATMPs with OD and NC by therapeutic area



ES ATMPs with OD and reimbursed in Spain by therapeutic area



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Detail of the 18 ATMPs with OD and MA (1/6)

	Brand name	Therapeutic area	Therapeutic indication	Company	MA Date	NC Date	Status/Date of SNS Reimbursement
1	**ABECMA**	Oncology	Treatment of adult patients with relapsed and refractory multiple myeloma who have received at least two prior therapies, including an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 antibody, and who have demonstrated disease progression during their most recent therapy.	Bristol Myers Squibb	18/08/2021	20/01/2022	Reimbursed 01/03/2024
2	**CARVYKTI**	Oncology	Treatment of adult patients with relapsed and refractory multiple myeloma who have received at least one prior therapy, including an immunomodulatory agent and a proteasome inhibitor, who have demonstrated disease progression during their most recent treatment, and who are eligible for lenalidomide.	Janssen-Cilag	25/05/2022	07/06/2022	Reimbursed 01/04/2025
3	**CASGEVY**	Hematology	• Treatment of transfusion-dependent β-thalassemia (TDT) in patients aged 12 years or older for whom hematopoietic stem cell (HSC) transplantation is appropriate and for whom a human leukocyte antigen (HLA)-compatible related HSC donor is not available. • Treatment of severe sickle cell disease (SCD) in patients 12 years of age or older with recurrent vaso-occlusive crises (VOCs) for whom hematopoietic stem cell (HSC) transplantation is appropriate and for whom a human leukocyte antigen (HLA)-compatible related HSC donor is not available.	Vertex Pharmaceuticals	09/02/2024	01/03/2024	Not reimbursed by resolution

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Detail of the 18 ATMPs with OD and MA (2/6)

	Brand name	Therapeutic area	Therapeutic indication	Company	MA Date	NC Date	Status/Date of SNS Reimbursement
4	**EBVALLO**	Other	As monotherapy for the treatment of adult and pediatric patients aged 2 years and older with relapsed or refractory Epstein-Barr virus-positive post-transplant lymphoproliferative disorder (EBV+ PTLD) who have received at least one prior treatment. For patients who have undergone solid organ transplantation, prior treatment includes chemotherapy, unless chemotherapy is inappropriate.	Pierre Fabre	16/12/2022	12/01/2023	Not reimbursed by resolution
5	**HEMGENIX**	Hematology	Treatment of severe and moderately severe hemophilia B in adult patients with no history of factor IX inhibitors.	CSL	20/02/2023	15/03/2023	Reimbursed 01/11/2024
6	**HOLOCLAR**	Sense organs	Treatment of adult patients with moderate to severe limbal stem cell deficiency (a condition defined by the presence of superficial corneal neovascularization in at least two corneal quadrants, with central corneal involvement and severe visual acuity impairment), whether unilateral or bilateral, resulting from ocular burns caused by physical or chemical agents. A minimum of 1 to 2 mm² of healthy limbus is required for the biopsy.	Holostem	17/02/2015	19/09/2015	Not reimbursed by resolution

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Detail of the 18 ATMPs with OD and MA (3/6)

	Brand name	Therapeutic area	Therapeutic indication	Company	MA Date	NC Date	Status/Date of SNS Reimbursement
7	KYMRIAH	Oncology	• Pediatric patients and young adults up to 25 years of age with refractory B-cell acute lymphoblastic leukemia (ALL), post-transplant relapse, or second or subsequent relapse. • Adult patients with diffuse large B-cell lymphoma (DLBCL) who have relapsed or are refractory to treatment after two or more lines of systemic therapy. • Adult patients with relapsed or refractory follicular lymphoma (FL) after two or more lines of systemic therapy.	Novartis	23/08/2018	17/09/2018	Reimbursed 01/01/2019
8	LIBMELDY	Nervous system	Treatment of metachromatic leukodystrophy (MLD) characterized by biallelic mutations in the arysulfatase A (ARSA) gene that lead to reduced ARSA enzyme activity: in children with late infantile or early juvenile forms, without clinical manifestations of the disease; in children with the early juvenile form, with early clinical manifestations of the disease, who are still able to walk independently and before the onset of cognitive impairment.	Orchard Therapeutics	17/12/2020	19/10/2021	Reimbursed 01/01/2025
9	LUXTURNA	Sense organs	Treatment of adult and pediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic mutations in RPE65 and who have sufficient viable retinal cells.	Novartis	22/11/2018	02/04/2019	Reimbursed 01/05/2021

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Detail of the 18 ATMPs with OD and MA (4/6)

	Brand name	Therapeutic area	Therapeutic indication	Company	MA Date	NC Date	Status/Date of SNS Reimbursement
10	ROCTAVIAN	Hematology	Treatment of severe hemophilia A in adult patients with no history of factor VIII inhibitors and no detectable antibodies to adeno-associated virus serotype 5 (AAV5).	BioMarin	24/08/2022	03/02/2023	Not reimbursed by resolution
11	STRIMVELIS	Immunomodulators	Treatment of patients with severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID) for whom a human leukocyte antigen (HLA)-matched related stem cell donor is not available.	Orchard Therapeutics	26/05/2016	No NC	-
12	TECARTUS	Oncology	• Treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL) following two or more lines of systemic therapy, including a Bruton's tyrosine kinase (BTK) inhibitor. • Treatment of adult patients aged 26 years and older with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL).	Gilead	14/12/2020	28/12/2020	Reimbursed 01/02/2024

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Detail of the 18 ATMPs with OD and MA (5/6)

	Brand name	Therapeutic area	Therapeutic indication	Company	MA Date	NC Date	Status/Date of SNS Reimbursement
13	**UPSTAZA**	Nervous system	Treatment of patients aged 18 months or older with a clinically, molecularly, and genetically confirmed diagnosis of L-aromatic amino acid decarboxylase (AADC) deficiency presenting with a severe phenotype.	PTC Therapeutics	18/07/2022	22/12/2022	Not reimbursed by resolution
14	**VUJYVEK**	Dermatology	Wound management in patients with epidermolysis bullosa dystrophica (EBD) with mutations in the collagen type VII alpha chain 1 (COL7A1) gene.	Krystal Biotech	23/04/2025	24/10/2025	Estudio o sin petición de financiación
15	**WASKYRA**	Hematology	Waskyra is indicated for the treatment of patients 6 months of age or older with Wiskott-Aldrich syndrome (WAS) who have a mutation in the WAS gene, for whom hematopoietic stem cell (HSC) transplantation is appropriate, and for whom a suitable human leukocyte antigen (HLA)-matched hematopoietic stem cell donor is not available.	Fondazione Telethon	09/01/2026	No NC	-

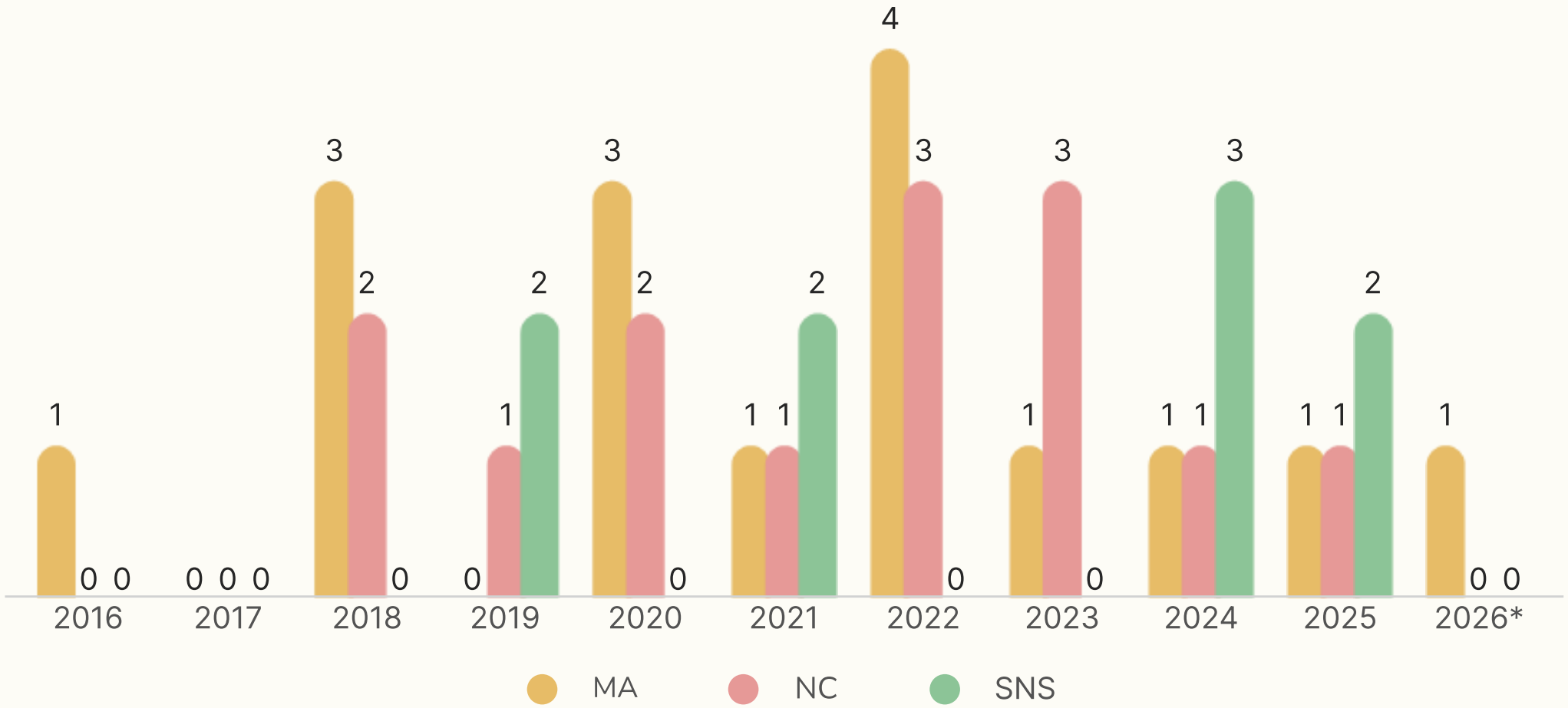
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Detail of the 18 ATMPs with OD and MA (6/6)

	Brand name	Therapeutic area	Therapeutic indication	Company	MA Date	NC Date	Status/Date of SNS Reimbursement
16	YESCARTA	Oncology	• Treatment of adult patients with diffuse large B-cell lymphoma (DLBCL) and high-grade B-cell lymphoma (HGBL) that relapses within 12 months of completion of first-line chemoimmunotherapy or is refractory to it. • Treatment of adult patients with relapsed or refractory (r/r) DLBCL and primary mediastinal large B-cell lymphoma (PMBCL) following two or more lines of systemic therapy. • Treatment of adult patients with relapsed or refractory follicular lymphoma (FL) following three or more lines of systemic therapy.	Gilead	23/08/2018	06/09/2018	Reimbursed 01/07/2019
17	ZEMCELPRO	Hematology	Treatment of adult patients with hematologic malignancies who require an allogeneic hematopoietic stem cell transplant following myeloablative conditioning and for whom no other suitable donor cells are available.	Cordex	25/08/2025	No NC	-
18	ZOLGENSMA	Musculoskeletal system	Treatment of patients with 5q spinal muscular atrophy (SMA) who have a biallelic mutation in the SMN1 gene and a clinical diagnosis of SMA type 1, or patients with 5q SMA who have a biallelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene.	Novartis	18/05/2020	28/07/2020	Reimbursed 01/12/2021

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Evolution of ATMPs by year (2016-2026*)



Note: The chart shows the situation of ATMPs year by year by indicators (MA, NC, and reimbursed by the SNS), the data are not cumulative year by year.
The ATMPs counted in each category (MA, NC, SNS) in the same year do not necessarily correspond to the same therapy.
All ATMPs analyzed in this slide have a valid OD.

*Data accumulated until April 30, 2026.

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Reimbursed Advanced Therapy Medicinal Products

As of April 30, 2026, there are 9 reimbursed ATMPs:

4 months
Average time from MA to NC

< 1 month
Median time from MA to NC

20 months
Average time from NC to reimbursement

25 months
Median time from NC to reimbursement

100 %
Approved with reimbursement conditions by the SNS

100 %
Monitored through the SEGUIMED procedure

89 %
Required to complete the pharmacoclinical protocol through VALTERMED

Note: All ATMPs analyzed in this slide have a valid OD.

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Detail of the 9 reimbursed ATMPs (1/2)

	Brand name	Therapeutic area	Company	Reimbursement date	Months from MA to reimbursement	CIPM Meetings	Reimbursement criteria[1]	Reported reimbursement price	Reimbursement conditions
1	ABECMA	Oncology	Bristol Myers Squibb	01/03/2024	26	13/12/2023 20/09/2023	a) and c) -	350.000,0 €	• Seguimed • Valtermed • Risk-sharing/Payment for results • Pharmacoclinical protocol
2	CARVYKTI	Oncology	Janssen-Cilag	01/04/2025	34	26/02/2025 29/01/2024 20/09/2023	a) and c) - -	420.000,0€	• Seguimed • Risk-sharing/Payment for results • Sales threshold
3	HEMGENIX	Hematology	CSL	01/11/2024	20	26/09/2024 24/04/2024 20/09/2023	a) and c) - -	2.800.000,0 €	• Seguimed • Valtermed • Risk-sharing/Payment for results • Sales threshold
4	KYMRIAH	Oncology	Novartis	01/01/2019	4	04/03/2020	Article 96.2	320.000,0 €	• Seguimed • Valtermed • Risk-sharing/Payment for results • Pharmacoclinical protocol • Provided to patients at no cost • Patient Registration • Supply by sales or age
5	LIBMELDY*	Nervous system	Orchard Therapeutics	01/01/2025	39	20/11/2024 26/09/2024 15/06/2023 02/03/2023	a) and c) - - -	2.875.000,0 €	• Seguimed • Valtermed • Spending cap • Risk-sharing/Payment for results

***LIBMELDY appears in BIFIMED with "reimbursement status: Yes for certain indications/conditions", however, in the "BIFIMED status" it appears as "H-DISCONTINUED DUE TO NOT MARKETING".**

Note: All ATMPs analyzed in this slide have a valid OD.

Source: BIFIMED (consulted in May 2026) and CIPM minutes (last published minutes: March 2026). Agreements of the Interministerial Commission on Drug Prices

In case of discrepancy between the information collected in the CIPM minutes and BIFIMED, the information contained in the latter shall prevail.

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Detail of the 9 reimbursed ATMPs (2/2)

	Brand name	Therapeutic area	Company	Reimbursement date	Months from MA to reimbursement	CIPM Meetings	Reimbursement criteria[1]	Reported reimbursement price	Reimbursement conditions
6	LUXTURNA	Sense organs	Novartis	01/05/2021	25	17/12/2020	a) and c)	345.000,0 €	• Seguimed • Valtermed • Spending cap • Pharmacoclinical protocol
7	TECARTUS	Oncology	Gilead	01/02/2024	38	23/12/2023 04/04/2022 16/12/2021	a) and c) - -	360.000,0 €	• Seguimed • Valtermed • Risk-sharing/Payment for results • Pharmacoclinical protocol • Refund for the difference between PVL and MSE
8	YESCARTA	Oncology	Gilead	01/07/2019	10	29/01/2024 23/11/2023	a) and c) -	327.000,0 €	• Seguimed • Valtermed • Risk-sharing/Payment for results • Pharmacoclinical protocol • Provided to patients at no cost • Patient Count
9	ZOLGENSMA	Musculoskeletal system	Novartis	01/12/2021	16	07/04/2021	a) and c)	1.945.000,0 €	• Seguimed • Valtermed • Risk-sharing/Payment for results • Pharmacoclinical protocol • Price-volume agreement

Note: All ATMPs analyzed in this slide have a valid OD.
Source: BIFIMED (consulted in May 2026) and CIPM minutes (last published minutes: March 2026). Agreements of the Interministerial Commission on Drug Prices In case of discrepancy between the information collected in the CIPM minutes and BIFIMED, the information contained in the latter shall prevail.

1 Criteria for reimbursement:

a) Severity, duration, and sequelae of the different pathologies for which they are indicated.
c) Therapeutic and social value of the medication and its incremental clinical benefit considering its cost-effectiveness ratio Article 96.2 of the Law on guarantees and rational use of medicines and health products

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Detail of ATMPs with unfavorable reimbursement resolution

	Brand name	Therapeutic area	Company	Unfavorable CIPM decision	Date of non-reimbursement	Non-reimbursement criteria[1]
1	CASGEVY	Hematology	Vertex Pharmaceuticals	19/11/2025 19/06/2025	01/01/2026	d)
2	EBVALLO	Other	Pierre Fabre	19/11/2025 26/02/2025 20/11/2024	01/04/2025	c) and d)
3	HOLOCLAR	Sense organs	Holostem	-	01/08/2016	-
4	ROCTAVIAN	Hematology	BioMarin	19/07/2024	01/10/2024	c) and d)
5	UPSTAZA	Nervous system	PTC Therapeutics	23/11/2023 11/05/2023	01/01/2024	d)

Note: All ATMPs analyzed in this slide have a valid OD.
Source: BIFIMED (consulted in May 2026) and CIPM minutes (last published minutes: March 2026). Agreements of the Interministerial Commission on Drug Prices In case of discrepancy between the information recorded in the CIPM minutes and BIFIMED, the information contained in the latter shall prevail.

1 Reasons for non-reimbursement:

c) Therapeutic and social value of the medicine and its incremental clinical benefit considering its cost-effectiveness ratio.
d) Rationalization of public expenditure allocated to pharmaceutical provision and budgetary impact on the National Health System.

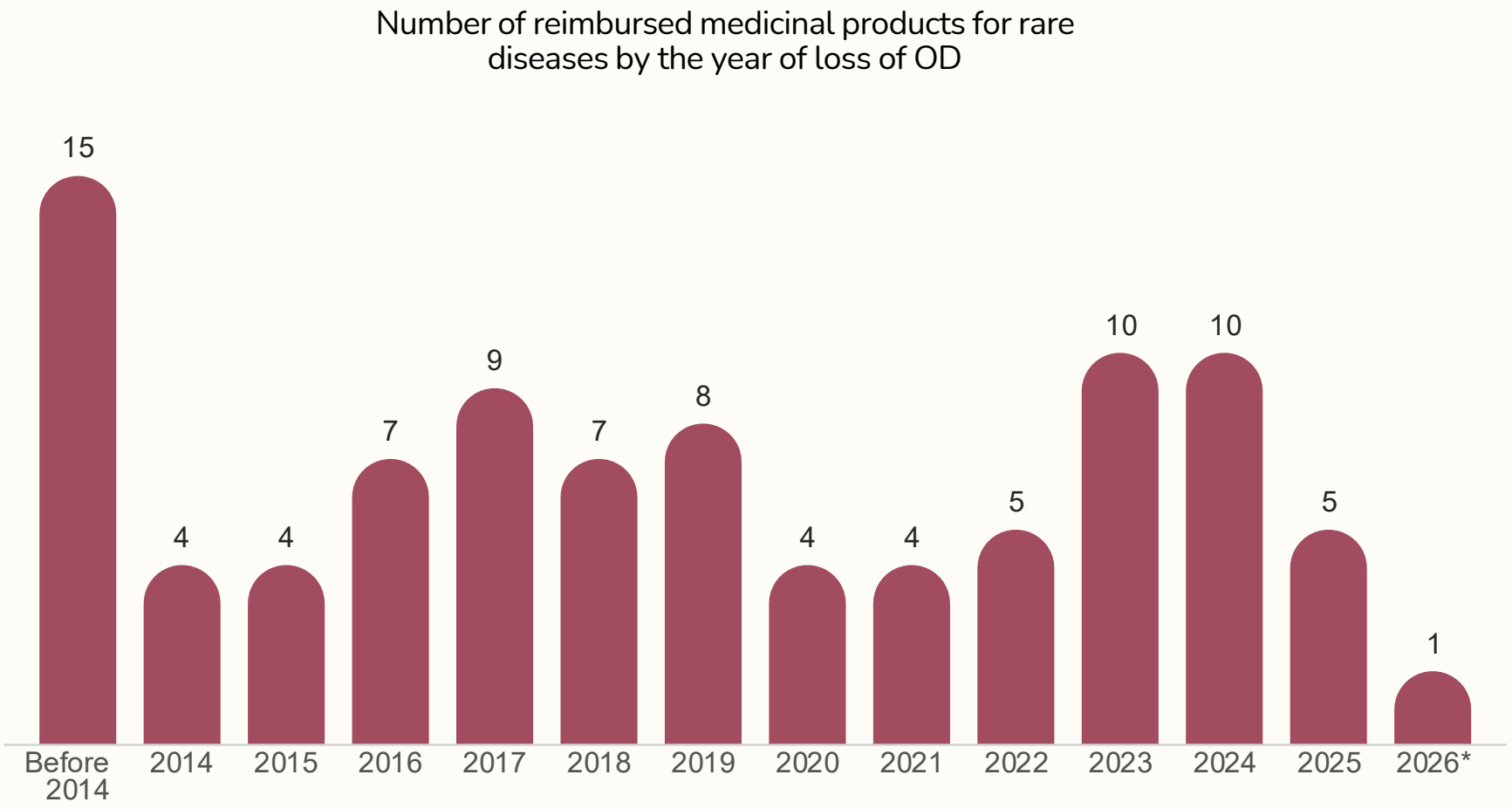
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Medicinal Products indicated for Rare Diseases

that have lost orphan designation

Reimbursed Medicinal products for rare diseases that have lost OD

93 medicinal products indicated for rare diseases, which have lost orphan designation between 2008 and 2026*, remain reimbursed by the SNS



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Source: Medicines that have lost orphan designation between 2008 and 2020 were obtained from the Orphanet analysis "List of medicinal products for rare diseases in Europe, August 2021". This list has been completed with medicines that lost orphan designation in 2021, 2022, 2023, 2024, 2025, and 2026.

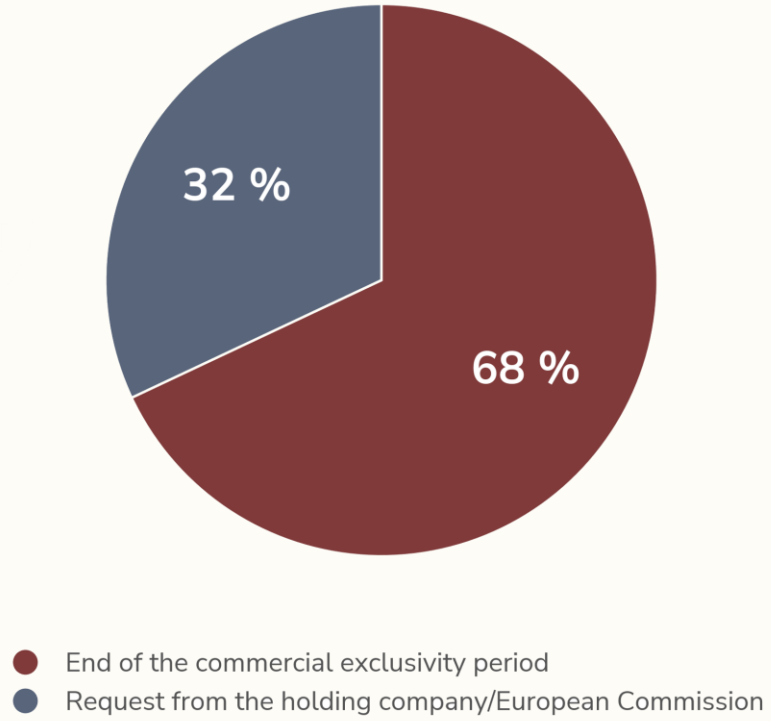
*Data accumulated until April 30, 2026.

Reimbursed Medicinal products for rare diseases that have lost OD

Of the 93 medicines indicated for rare diseases that have lost orphan designation but are still reimbursed by the SNS:

- **32% (30)** lost orphan designation at the request of the marketing authorization holder or by a decision of the European Commission.
- **68% (63)** was due to the expiration of the established period of commercial exclusivity.

Reason for loss of OD



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Source: The medicines that have lost orphan designation between 2008 and 2020 were obtained from the Orphanet analysis "List of medicinal products for rare diseases in Europe, August 2021". This list has been completed with the medicines that lost orphan designation in 2021, 2022, 2023, 2024, 2025, and 2026.


Summary:
Key facts

01 During the first four months of 2026:

- **10 new medicinal products with OD** and brand name.
- **7 orphan medicinal products** have obtained marketing authorization in the EU.
- **6 orphan medicinal products** have obtained National Code.
- **3 orphan medicinal products** have been reimbursed by Spain's National Health System.

02 As of April 30, 2026, there is a total of:

- **230 medicines with OD and brand name.**
- 163 orphan medicinal products with European marketing authorization.
- 141 orphan medicinal products with a National Code in Spain, 87% of those with MA.
- 106 orphan medicinal products reimbursed by the SNS, representing 65% of products with MA and 75% of those with NC.

03 Orphan Medicinal Products approval conditions:

- According to the type of approval, 44 (27%) of the orphan medicinal products authorized up to April 30, 2026, have been approved conditionally or under exceptional circumstances, while the rest correspond to standard approvals.
- Regarding special approval conditions, more than half of the OMPs authorized in Europe and/or Spain are subject to additional monitoring: 104 (64%) in the case of those with MA, 90 (64%) of those with NC, and 65 (61%) of those reimbursed in Spain.
- Of the 20 orphan medicinal products with conditional approval and NC, 15 have been reimbursed (75%), and of the 17 orphan medicinal products with NC approved under exceptional circumstances, 12 (71%) are reimbursed.

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04 Orphan Medicinal Products with MA pending arrival in Spain:

- Currently, 13% of the medicines with MA have not yet arrived in Spain (22) and, of these, 45% received MA more than a year ago.

05 Orphan Medicinal Products reimbursed between January and April 2026:

- **3 products have obtained reimbursement between January 1 and April 30, 2026.**
- The average waiting time from obtaining the National Code to reimbursement by the SNS has been 22 months.
- **No OMP has received reimbursement within the first year after obtaining NC, and no orphan medicinal product has managed to obtain reimbursement at its first CIPM meeting.**
- **Only 1 OMP took more than two years to be reimbursed after obtaining NC, and 2 attended at least four CIPM meetings before receiving reimbursement.**
- 100% of the medicines reimbursed during the first four months of 2026 have conditional reimbursement and 100% have restricted reimbursement (based on the indications approved by EMA).

06 Non-reimbursed treatments:

- **35 orphan medicinal products with NC are not reimbursed, either because:**
  - they are under study or without a reimbursement request (11); of these, 100% have been waiting less than a year for a favorable reimbursement resolution and none have been waiting more than three years for a favorable reimbursement resolution;
  - or because they have been excluded by resolution (24).

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07 Advanced Therapy Medicinal Products for rare diseases:

- As of April 30, 2026, 73% of the 67 ATMPs for rare diseases do not have a marketing authorization (MA). Of these, 41 are included in the EMA PRIME program, 3 are under evaluation by the CHMP, and 5 are academic ATMPs.
- **18 ATMPs have orphan designation and marketing authorization, of which 28% have oncological indication and 28% are hematological agents.**
- Of those 18 ATMPs, 15 already have a National Code (NC). By therapeutic area, 33% are oncological and 20% are hematological agents.
- **9 ATMPs are reimbursed by the SNS and most of them have oncological indication (56%). Likewise, 100% of these treatments are subject to monitoring through the SEGUIMED procedure and 89% to pharmacological protocol through VALTERMED.**
- Of the 6 ATMPs excluded from SNS reimbursement:

  - 5 have unfavorable reimbursement resolutions between 2016 and the first four months of 2026.
  - 1 are under study or without reimbursement request.

08 Treatments that have lost orphan designation but are still reimbursed by the SNS:

- **93 medicines indicated for rare diseases have lost orphan designation but are still reimbursed by the SNS.**
- Of those medicinal products:

  - 32% have lost the OD at the request of the marketing authorization holder or by a decision of the European Commission.

  - 68% have lost the OD due to the expiration of the established period of commercial exclusivity.

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Bibliography

01 Medicines with orphan designation from the European Medicines Agency

02 Community Register of Orphan Medicinal Products

03 CIMA (AEMPS)

04 BIFIMED (Spain's Ministry of Health)

05 Agreements Interministerial Commission on Medicine Prices (CIPM)

06 PRIME Program: Priority Medicines of the European Medicines Agency

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Acronyms

- **MA:** European marketing authorisation by centralised procedure
- **AEMPS:** Spanish Agency for Medicines and Medical Devices
- **BIFIMED:** Search engine for information on the reimbursement situation of medicines in Spain
- **CHMP:** Committee for Medicinal Products for Human Use of EMA
- **CIPM:** Interministerial Commission on Medicines Prices in Spain
- **NC:** National Code
- **COMP:** EMA's Committee for Orphan Medicinal Products
- **EMA:** European Medicines Agency
- **OMPs:** Orphan Medicinal Products
- **ATMPs:** Advanced Therapy Medicinal Products
- **OD:** Orphan Designation
- **PRIME:** Priority Medicines program by EMA
- **SEGUIMED:** Monitoring of medicine supply in the national market of Spain
- **SNS:** Spain's National Health System
- **EU:** European Union
- **VALTERMED:** Information System for determining the Therapeutic Value in Real Clinical Practice of Medicines with High Health and Economic Impact in the Spanish National Health System

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