Orphan medicinal products (OMPs) in Spain*

Market access report

JAN-AUG

2026

aelmhu



*Analysis of all OMPs with a brand name that have a valid European orphan designation as of August 31, 2026.

elaborado por:
bio innova Consulting

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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 August 31, 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, **231 orphan medicinal products (OMPs) with a valid OD as of August 31, 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Next, it has been analyzed which of these orphan medicinal products have been withdrawn (or have not yet been included) from the European Commission's Community Register of Orphan Medicinal Products for marketing in the European Union **(n=67)**.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 authorisation (MA) as of August 31, 2026**, in the European Union is obtained **(n=164)**.

03

The third step has been to identify which of **those orphan medicinal products with a brand name that have a marketing authorisation 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=105)**. The first registration date has been taken as the reimbursement date.

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Notes:

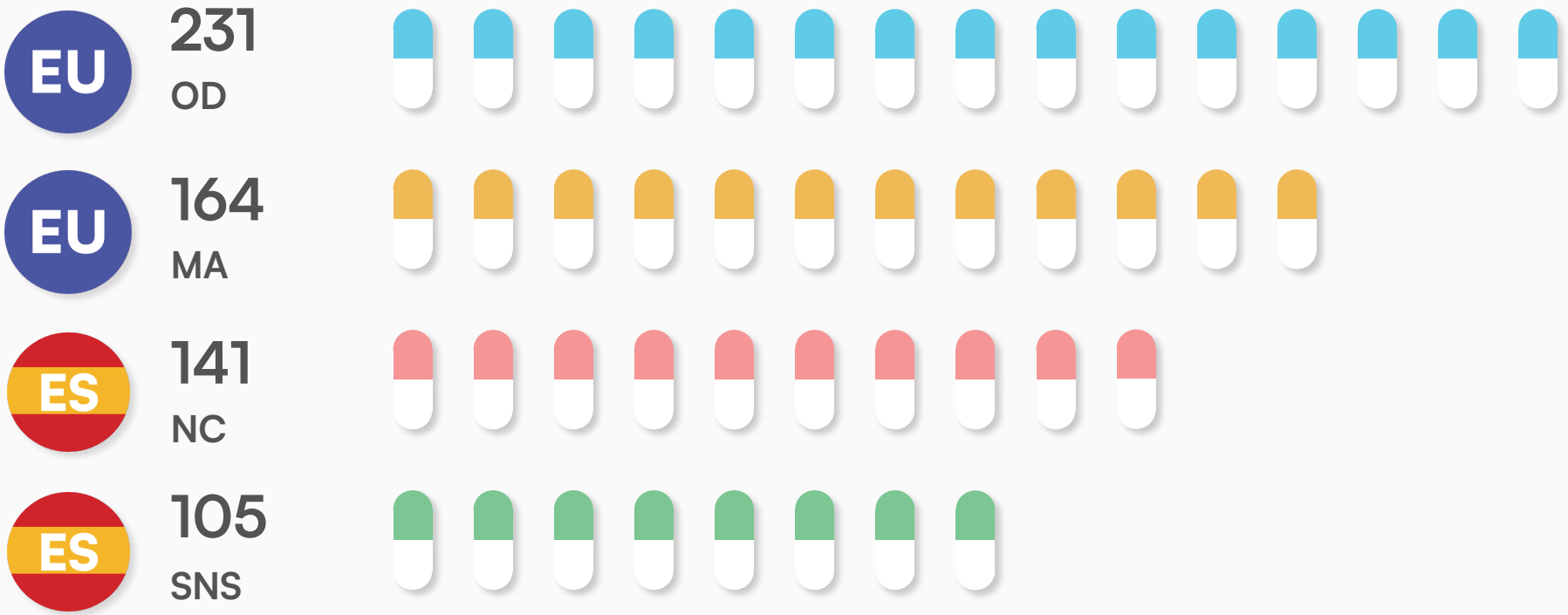
1. Because the data cannot be obtained from any public source, this document does not include those OMPs that are available in our country under specific circumstances and special authorisations provided for in Royal Decree 1015/2009 (compassionate use, use under conditions other than those authorised, or access to foreign medicines).
2. The company listed for each OMP throughout this report generally corresponds to the marketing authorisation 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*



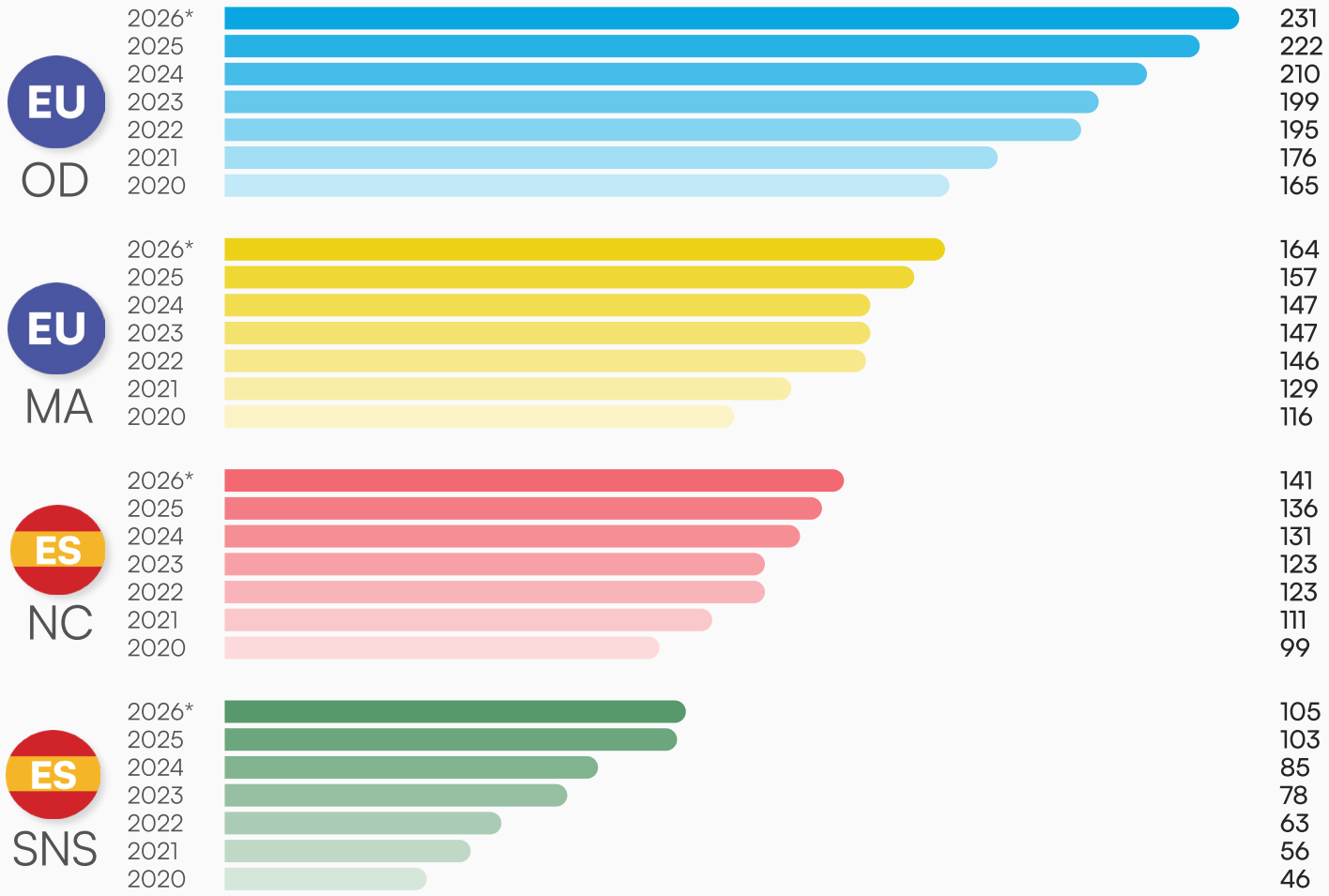
Notes:

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

*Data accumulated until August 31, 2026.

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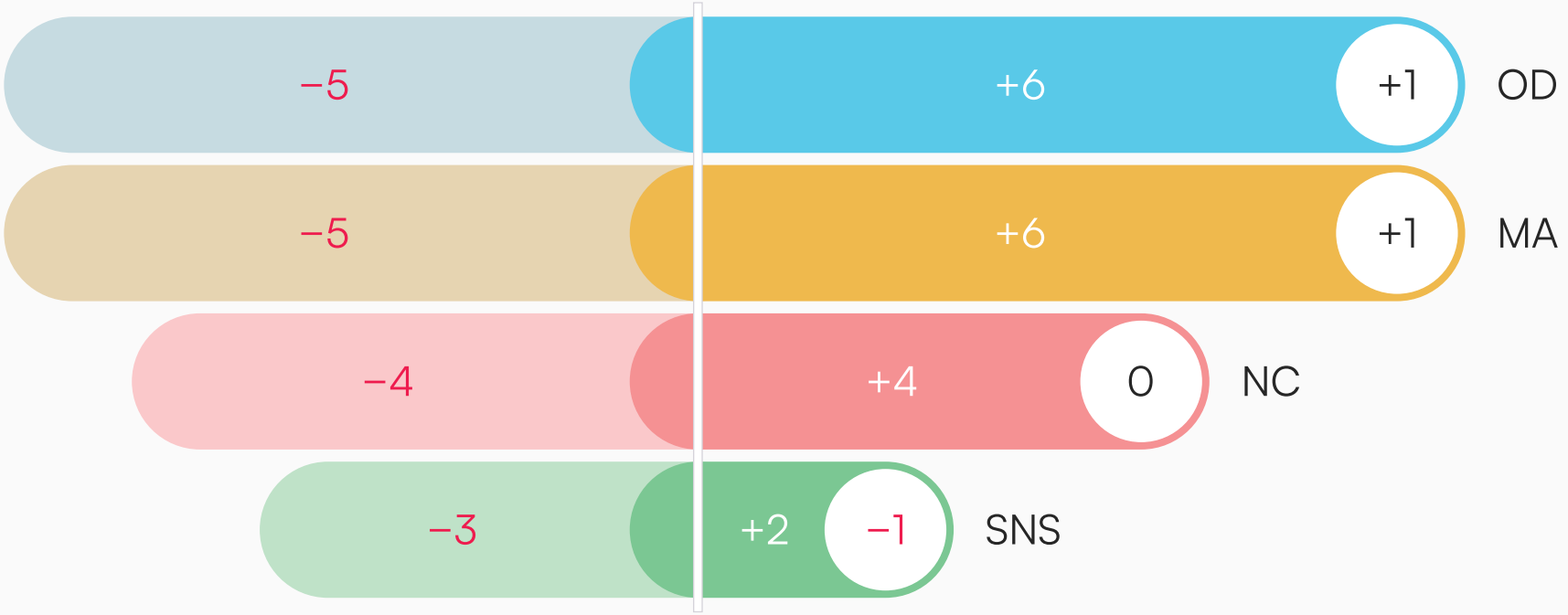
Variation of indicators between 2020 and 2026*



*Data accumulated until August 31, 2026.

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Variation of indicators: 2nd vs. 1st four-month period of 2026*



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*Data accumulated until August 31, 2026.

Detail of the variation of orphan designations

	2nd vs. 1st four-month period 2026	OMPs	Company	Comments
EU OD	+ 6 OMPs obtained OD	DEQTYNET ISEMBYLD LYNAVOY ORBLID QEZZAQAR YARTEMLEA	CIS Bio Scholar Rock GSK Delbert CATS Consultants Omeros	No MA 03/08/2026 No MA 13/08/2026 CHMP+ 23/07/2026 No MA 21/05/2026 CHMP- 23/07/2026 Under review
	- 5 OMPs lost OD	ALIQOPA DARZALEX DELTYBA GALAFOLD ORDSPONO	Bayer Janssen-Cilag Otsuka Amicus Regeneron	Lost OD Lost OD Lost OD Lost OD Lost OD

*Data accumulated until August 31, 2026.

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Detail of the variation of marketing authorisations

	2nd vs. 1st four-month period 2026	OMPs	Company	Comments
EU MA	+ 6 OMPs obtained MA	DAYBU IMDYLLTRA JOENJA REDEMPLO VIJOICE ZEPZELCA	Acadia Amgen Pharming Technologies Arrowhead Pharmaceuticals Novartis PharmaMar	MA 20/08/2026 MA 29/05/2026 MA 21/05/2026 MA 19/06/2026 MA 15/07/2026 MA 29/05/2026
	- 5 OMPs lost OD	DARZALEX DELTYBA GALAFOLD ORDSPONO NATPAR	Janssen-Cilag Otsuka Amicus Regeneron Takeda	Lost OD Lost OD Lost OD Lost OD Lost OD

*Data accumulated until August 31, 2026.

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Detail of the variation of national codes

	2nd vs. 1st four-month period 2026	OMPs	Company	Comments
ES NC	+ 4 OMPs obtained NC	IMDYLLTRA REDEMPLO SEPHIENCE ZEPZELCA	Amgen Arrowhead Pharmaceuticals PTC Therapeutics PharmaMar	NC 29/06/2026 NC 07/07/2026 NC 12/06/2026 NC 05/06/2026
	- 4 OMPs lost OD	DARZALEX DELTYBA GALAFOLD NATPAR	Janssen-Cilag Otsuka Amicus Takeda	Lost OD Lost OD Lost OD Lost OD

*Data accumulated until August 31, 2026.

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Detail of the variation of reimbursed OMPs

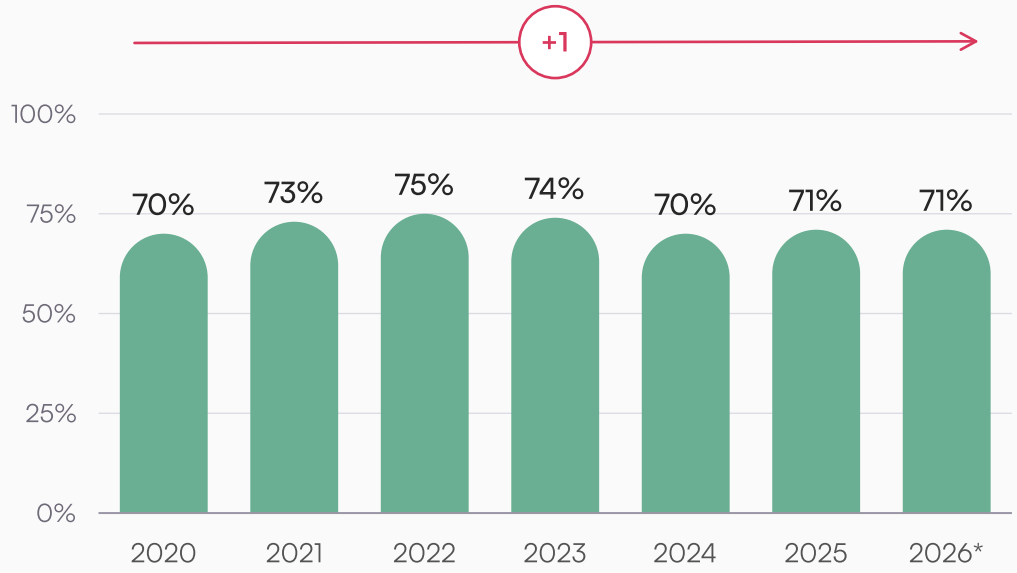
	2nd vs. 1st four-month period 2026	OMPs	Company	Comments
ES SNS	+ 2 OMPs obtained reimbursement	ALYFTREK MYALEPTA	Vertex Pharmaceuticals Chiesi	SNS 01/06/2026 SNS 01/08/2026
	- 3 OMPs lost OD	DARZALEX DELTYBA GALAFOLD	Janssen-Cilag Otsuka Amicus	Lost OD Lost OD Lost OD

*Data accumulated until August 31, 2026.

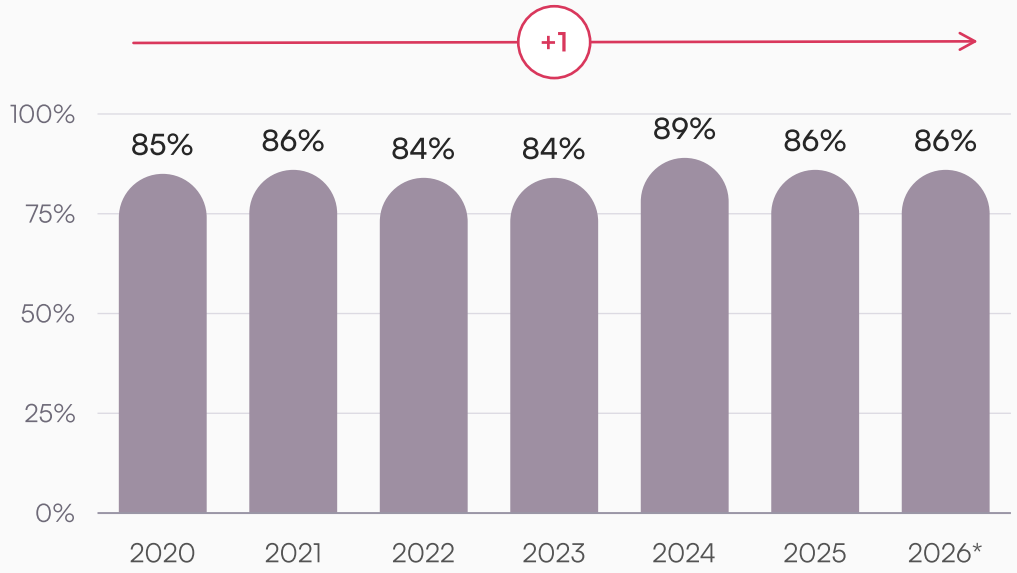
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Comparison between the indicators

% of OMPs with OD and MA



% of OMPs with MA and NC

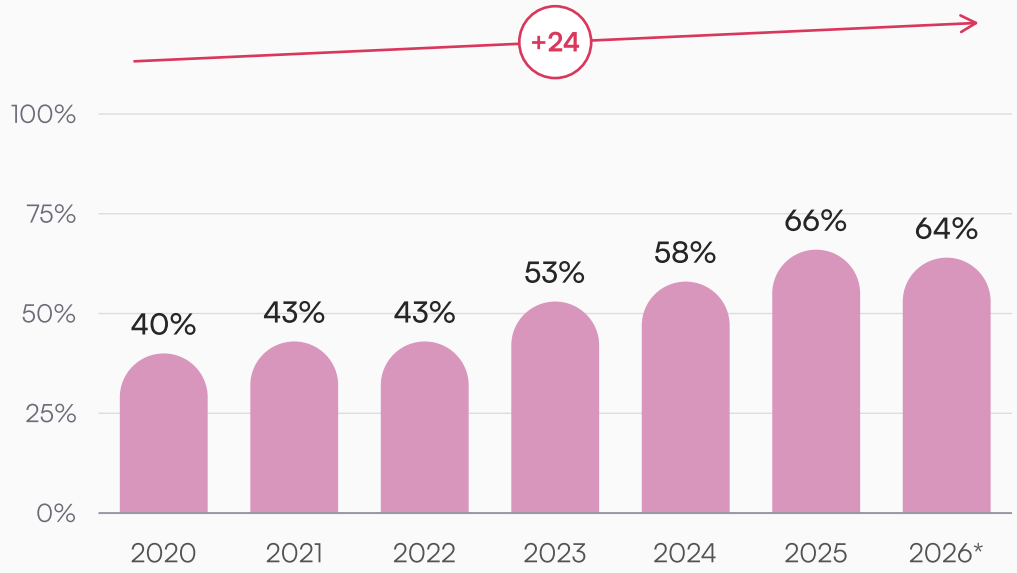


*Data accumulated until August 31, 2026.

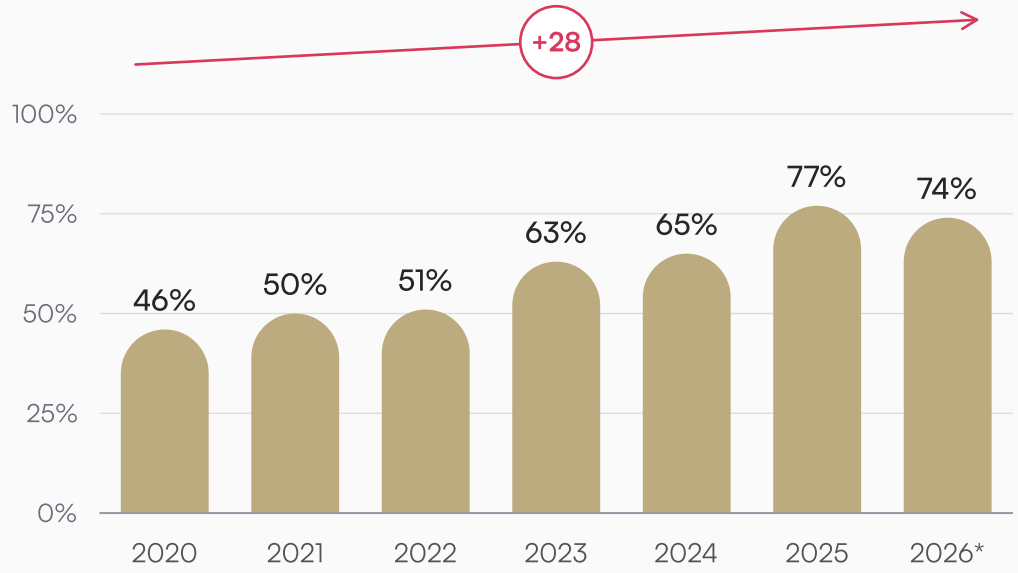
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Comparison between the indicators

% of OMPs with MA and reimbursed by the SNS



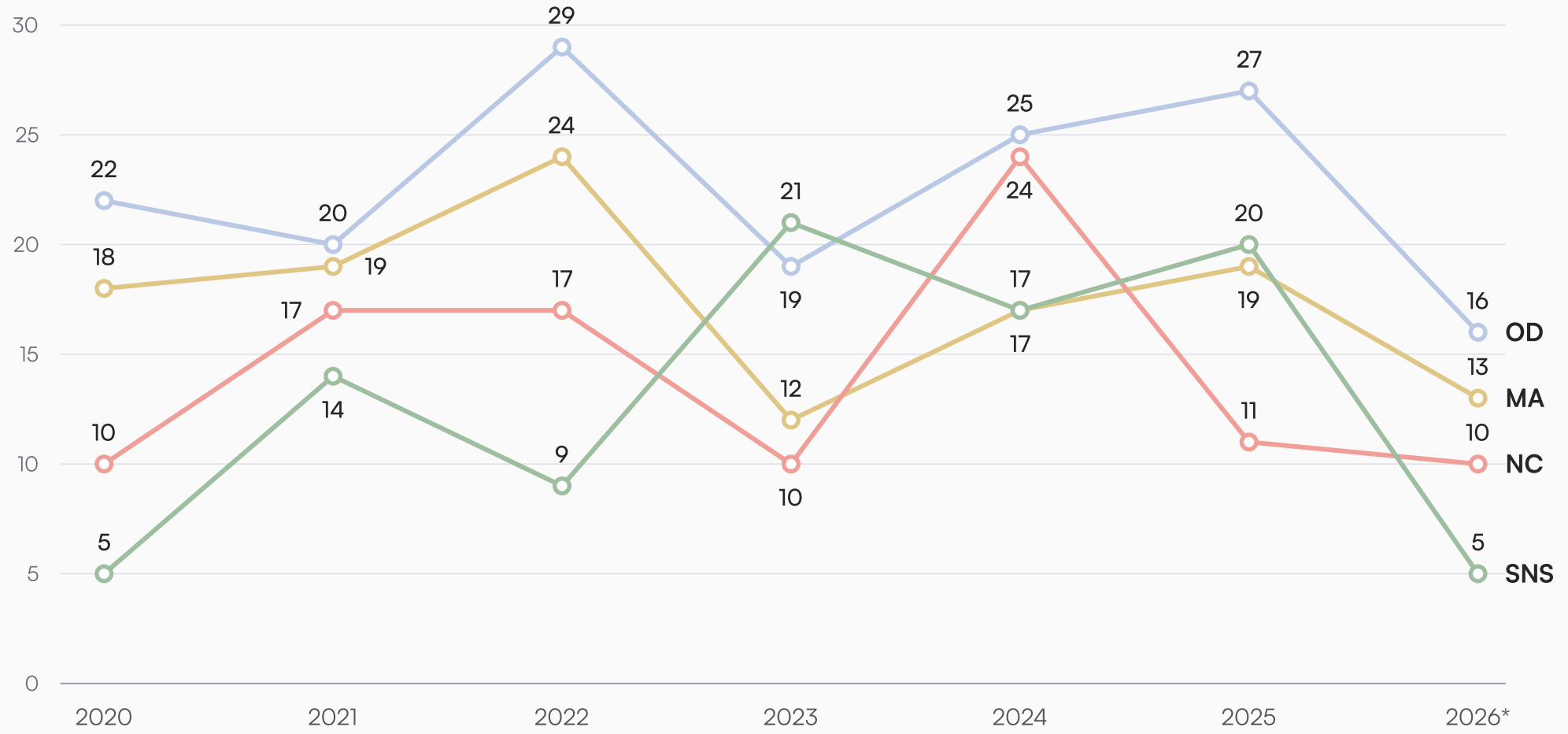
% of OMPs with NC and reimbursed by the SNS



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*Data accumulated until August 31, 2026.

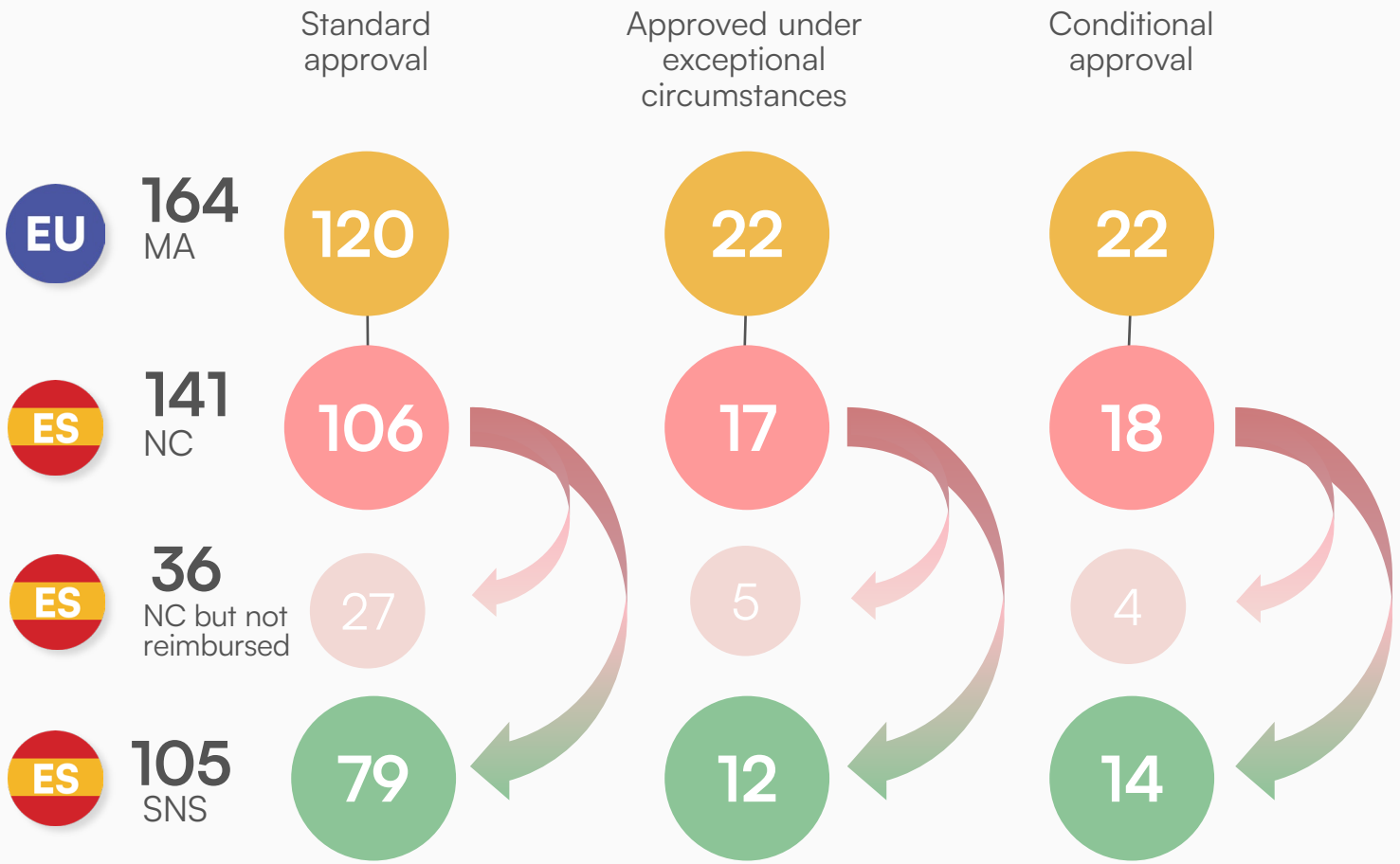
Summary of indicators by year



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*Data accumulated until August 31, 2026.

Type of approval for orphan medicinal products*



*Data accumulated until August 31, 2026.

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Special conditions associated with the approval of OMPs*

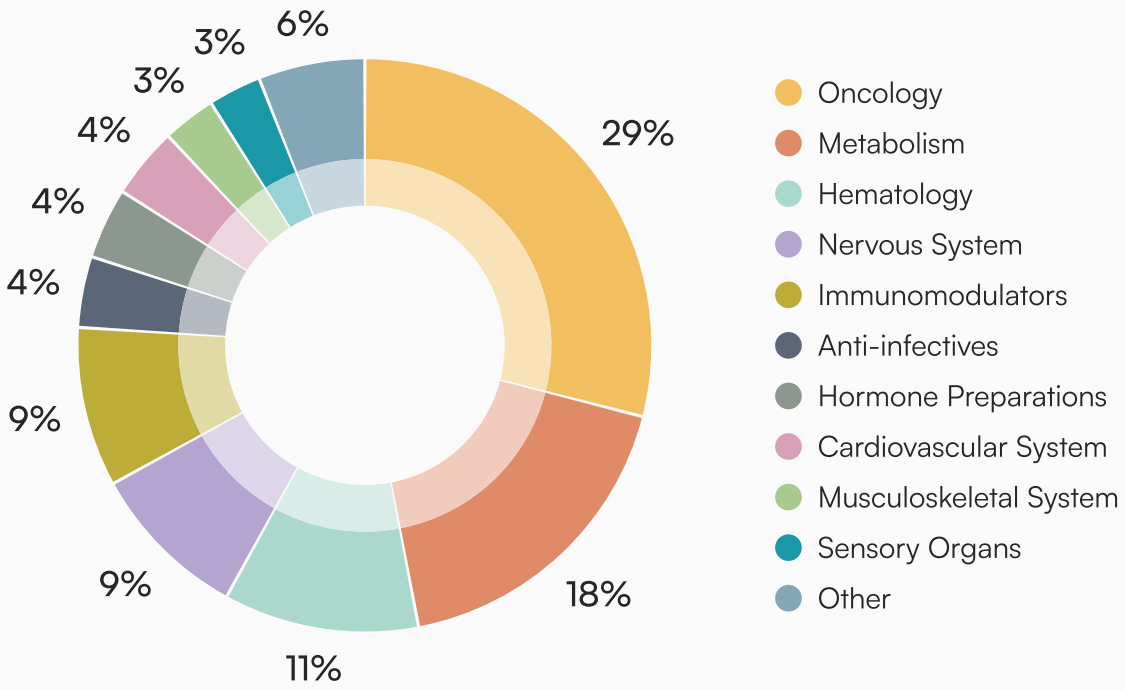


*The same orphan medicinal product may be subject to more than one special condition simultaneously. For this reason, the sum of "accelerated assessment," "additional monitoring," and "without any specification" does not necessarily match the total OMPs. Data accumulated until August 31, 2026.

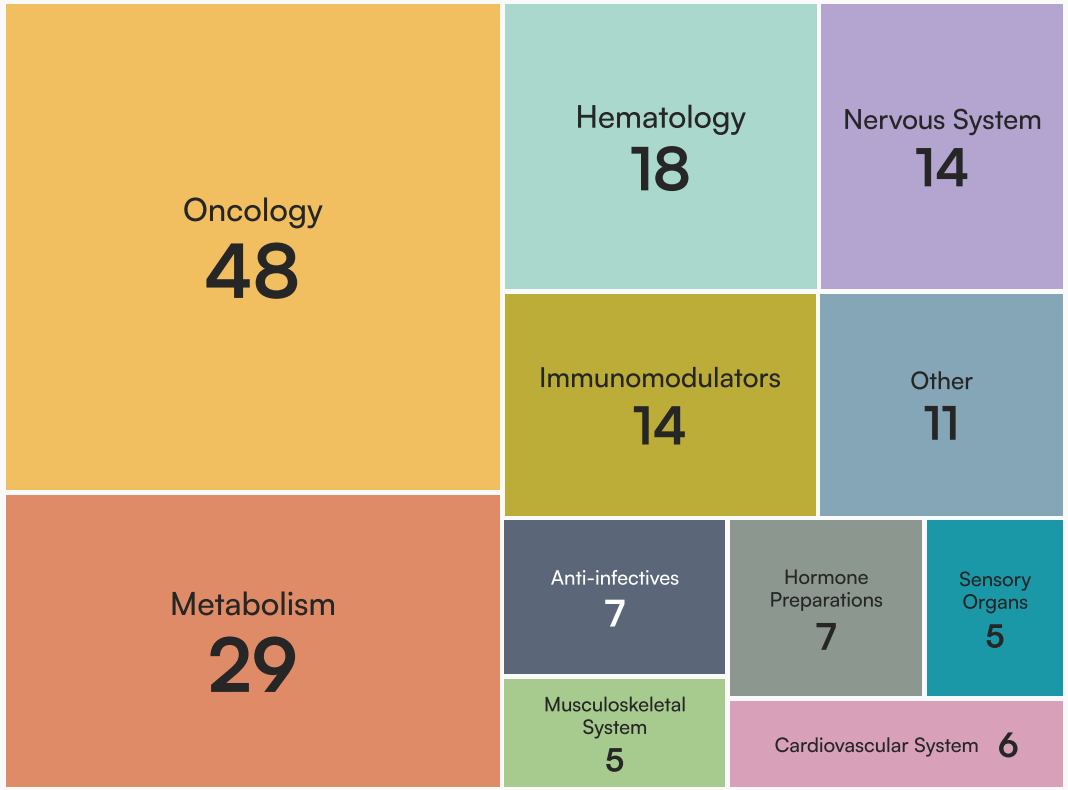
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Orphan Medicinal Products with marketing authorisation

By therapeutic area

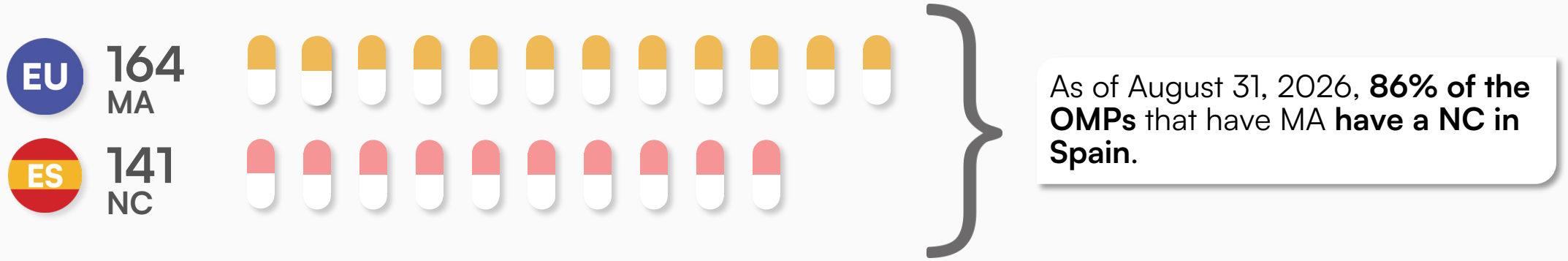


Absolute values



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Orphan Medicinal Products with MA vs. NC*



As of August 31, 2026, **86% of the OMPs** that have MA **have a NC in Spain.**

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*Data accumulated until August 31, 2026.

Detailed information of the 23 OMPs with MA without NC (1/2)

	Brand name	Company	MA date	Waiting months (MA — 31/08/2026)	Therapeutic area	ATMP*
1	AQNEURSA	IntraBio	19/01/2026	7	Nervous System	
2	ARIKAYCE	Insmed	27/10/2020	71	Anti-infectives	
3	DAYBU	Acadia	20/08/2026	0	Nervous System	
4	DOVPRELA	Mylan	31/07/2020	74	Anti-infectives	
5	EKTERLY	KalVista	17/09/2025	12	Hematology	
6	EMCITATE	Rare Thyroid Therapeutics International	12/02/2025	19	Hematology	
7	HYFTOR	PlusUltra	15/05/2023	40	Dermatology	
8	JOENJA	Pharming Technologies	21/05/2026	3	Immunomodulators	
9	KYGEVVI	UCB Pharma	26/03/2026	5	Metabolism	
10	MAAPLIV	Recordati	28/07/2025	13	Metabolism	
11	NULIBRY	Comharsa	15/09/2022	48	Metabolism	
12	OJEMDA	Ipsen	20/04/2026	4	Oncology	

*ATMP: advanced therapy medicinal product

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Detailed information of the 23 OMPs with MA without NC (2/2)

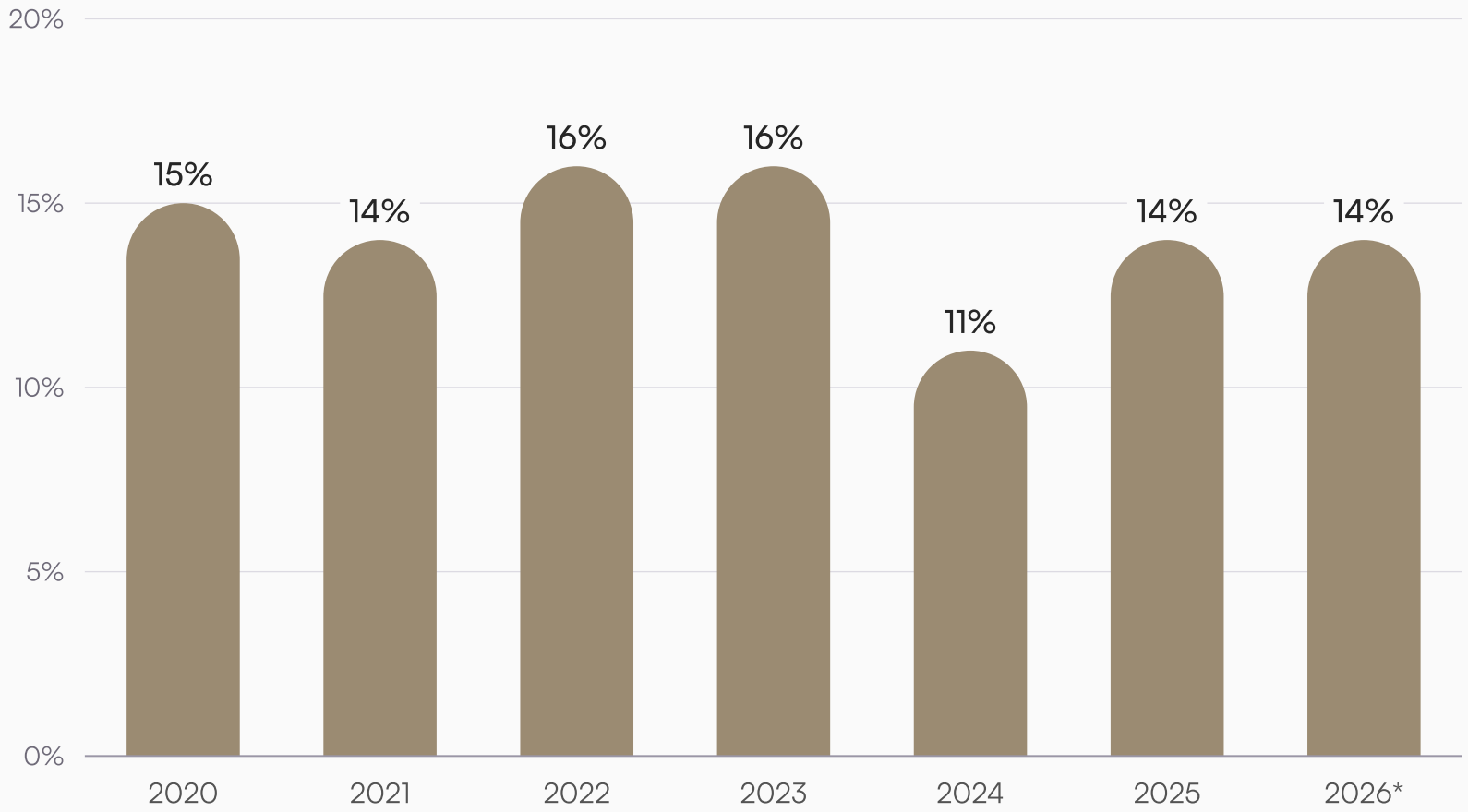
	Brand name	Company	MA date	Waiting months (MA — 31/08/2026)	Therapeutic area	ATMP*
13	PALSONIFY	Crinetics Pharmaceuticals	23/04/2026	4	Hormone Preparations	
14	PYRUKYND**	Avanzanite Bioscience	09/11/2022	46	Hematology	
15	RYTELO	Geron	07/03/2025	18	Oncology	
16	SKYTROFA	Ascendis Pharma	11/01/2022	56	Hormone Preparations	
17	STRIMVELIS	Fondazione Telethon	26/05/2016	125	Immunomodulators	●
18	VIJOICE	Novartis	15/07/2026	2	Immunomodulators	
19	WASKYRA	Fondazione Telethon	09/01/2026	8	Hematology	●
20	XOLREMDI	X4 Pharmaceuticals	27/04/2026	4	Immunomodulators	
21	ZEMCELPRO	Cordex	25/08/2025	12	Hematology	●
22	ZIIHERA	Jazz Pharmaceuticals	27/06/2025	14	Oncology	
23	ZOKINVY	EigerBio	18/07/2022	50	Metabolism	

*ATMP: advanced therapy medicinal producto

** PYRUKYND, marketed in Spain by Avanzanite Bioscience, has had a national marketing authorisation since 05/06/2026. It was not included in this report due to a synchronisation error in the CIMA database, which was identified after its publication and has since been corrected in the official source. This information will be duly reflected in the next annual edition of the access report.

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Percentage of orphan medicinal products with MA without NC

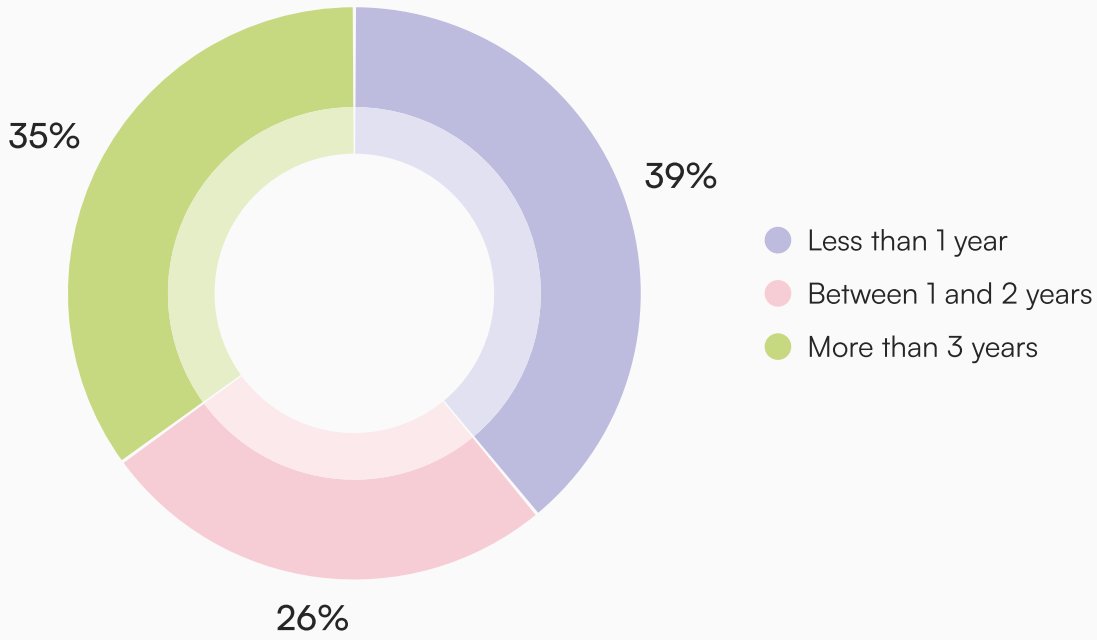


*Data accumulated until August 31, 2026.

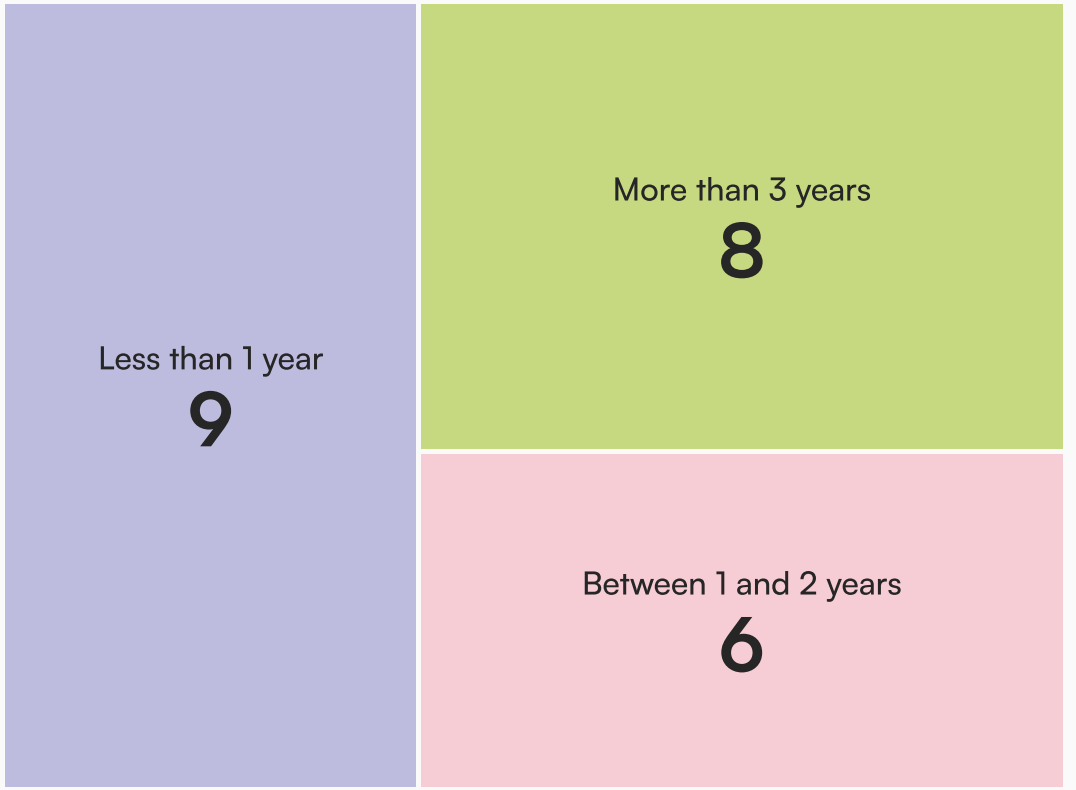
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Orphan Medicinal Products with MA without NC by waiting years

By waiting years



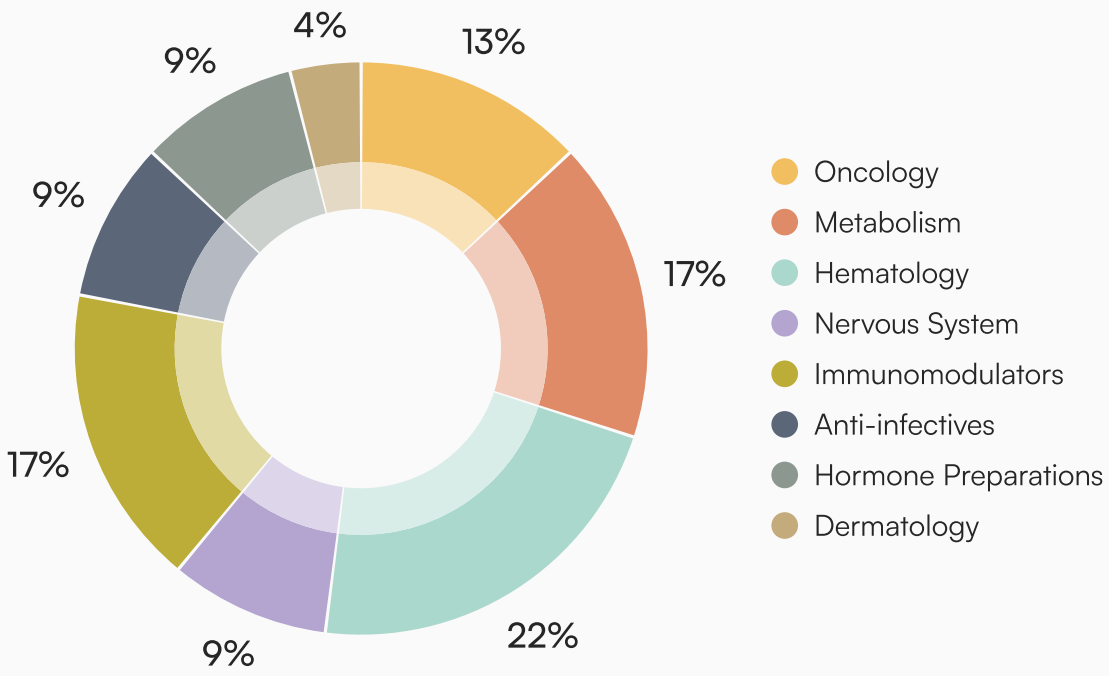
Absolute values



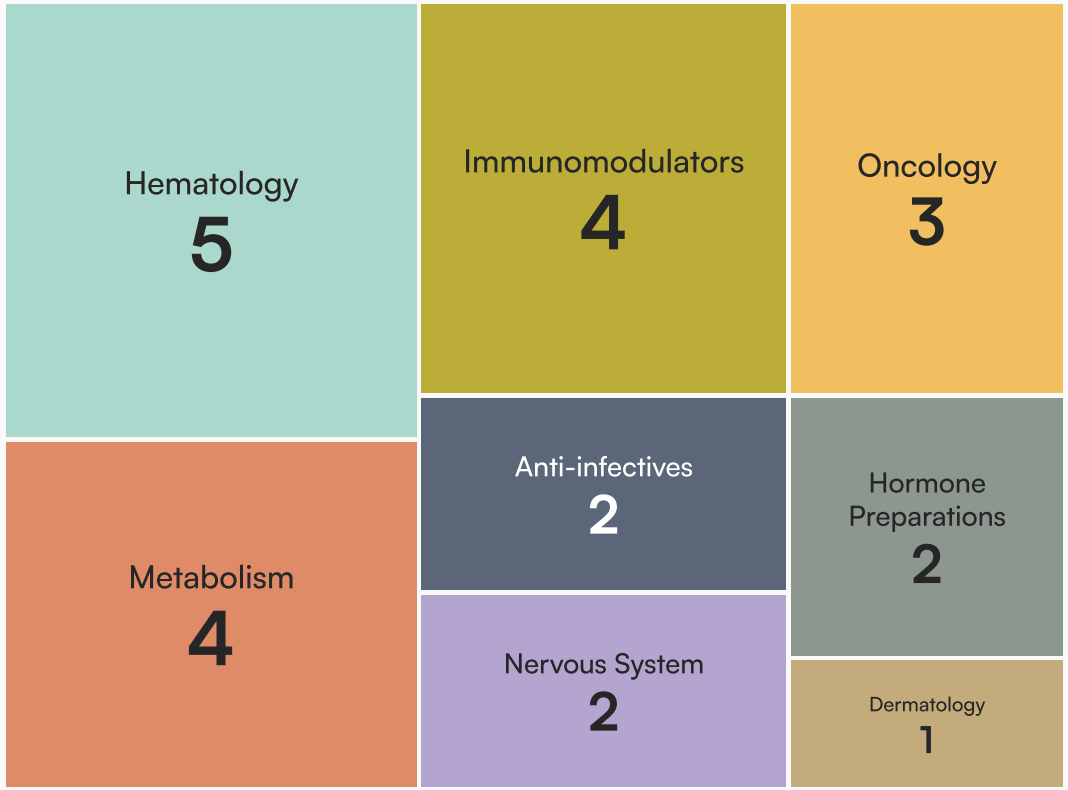
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Orphan Medicinal Products with MA without NC by therapeutic area

By therapeutic area



Absolute values



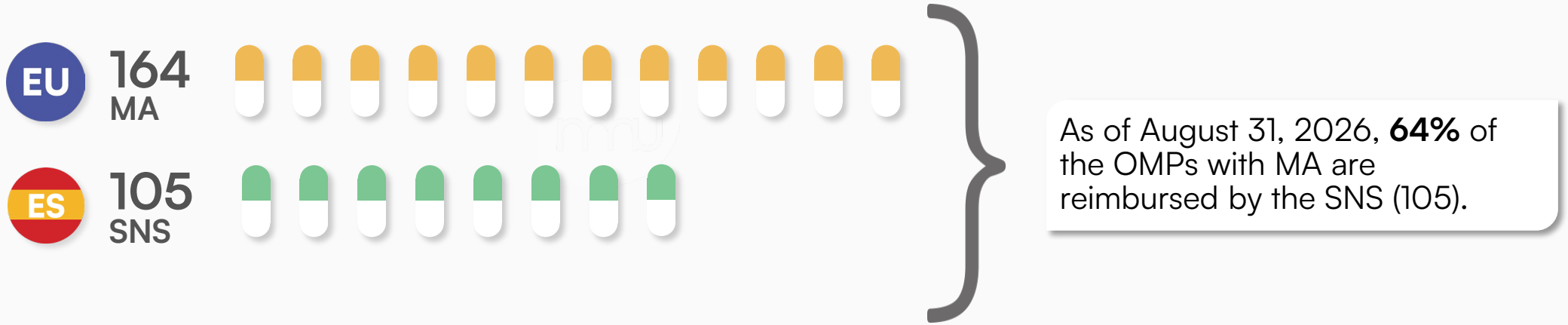
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Orphan Medicinal Products

Spain



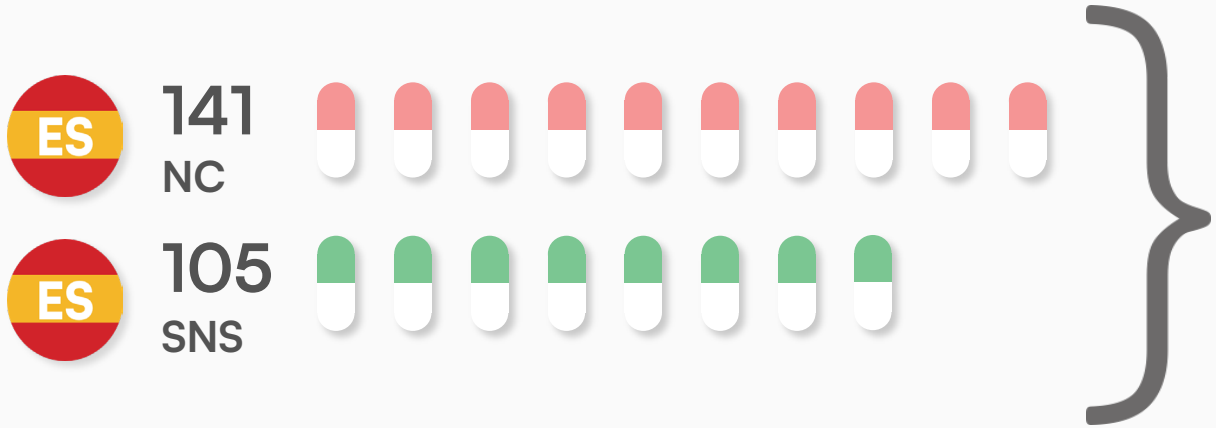
Orphan Medicinal Products with MA vs. reimbursed



As of August 31, 2026, **64%** of the OMPs with MA are reimbursed by the SNS (105).

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Comparison of OMPs with NC vs. reimbursed by the SNS

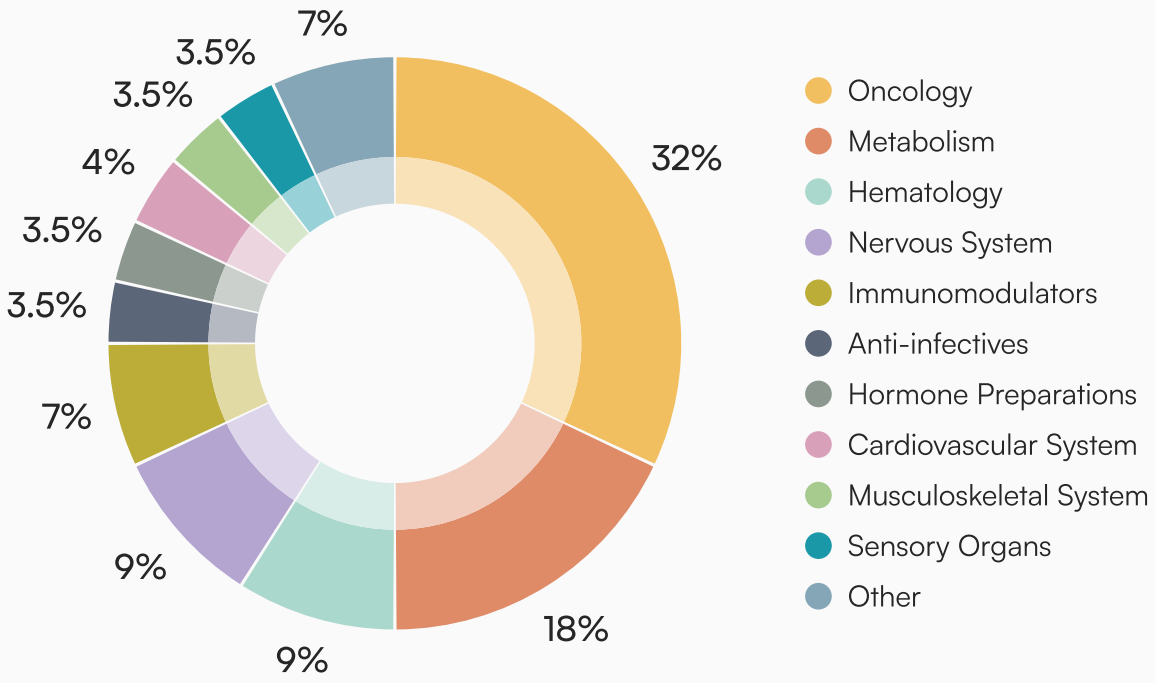


As of August 31, 2026, **74% of the OMPs** that have arrived in Spain **are currently reimbursed** by the SNS.

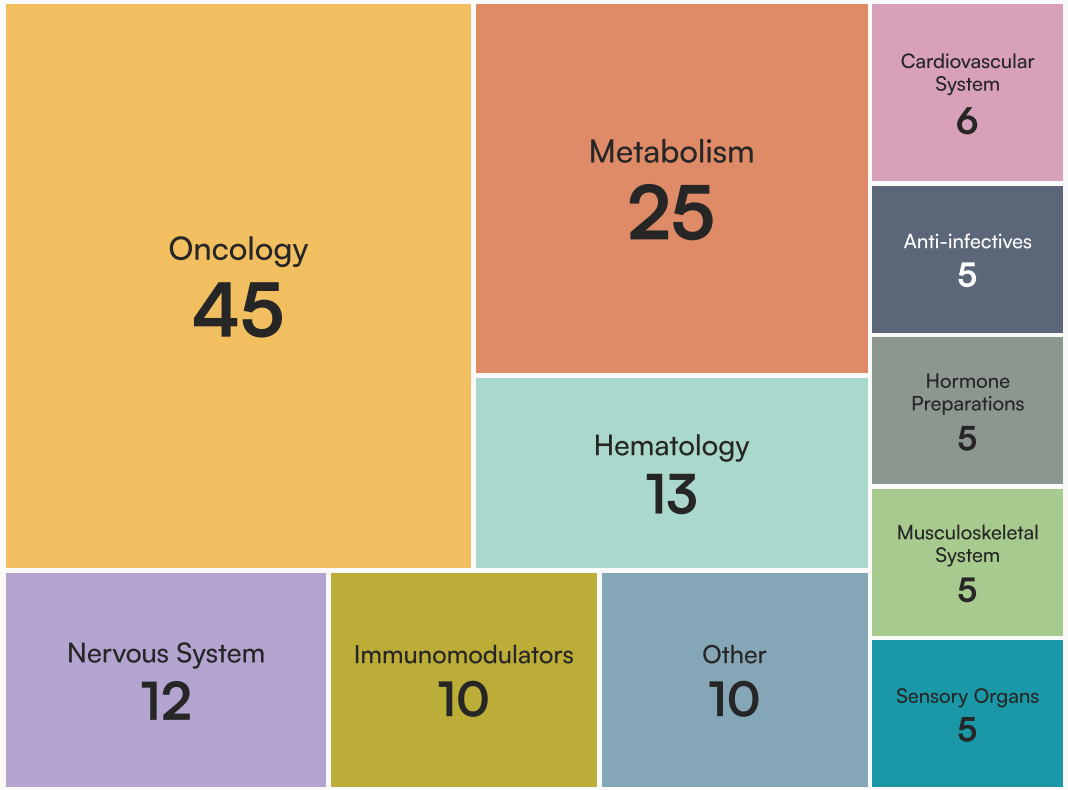
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OMPs with NC by therapeutic area

By therapeutic area



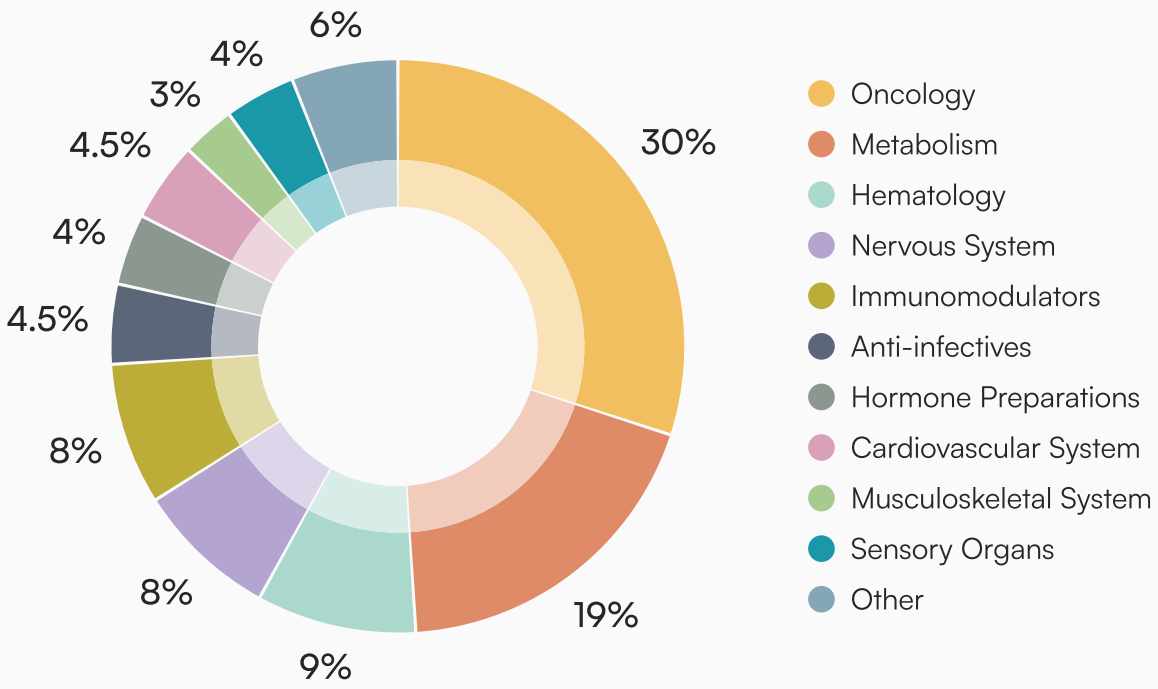
Absolute values



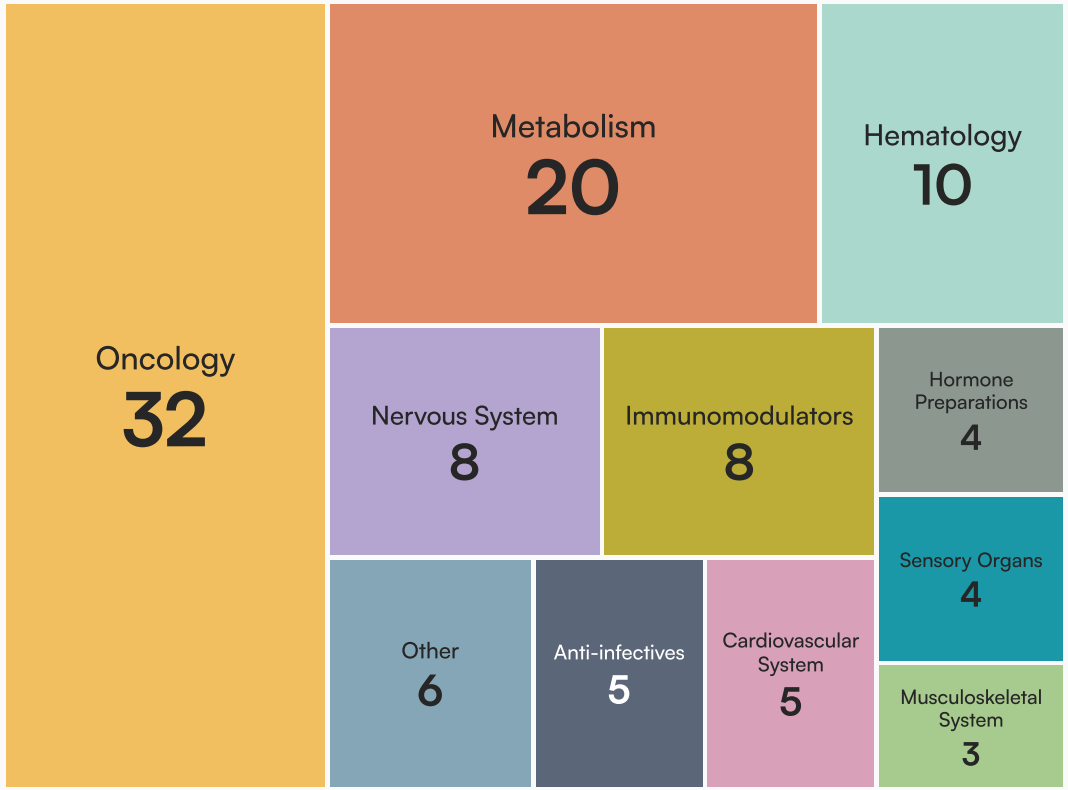
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OMPs reimbursed by therapeutic area

By therapeutic area

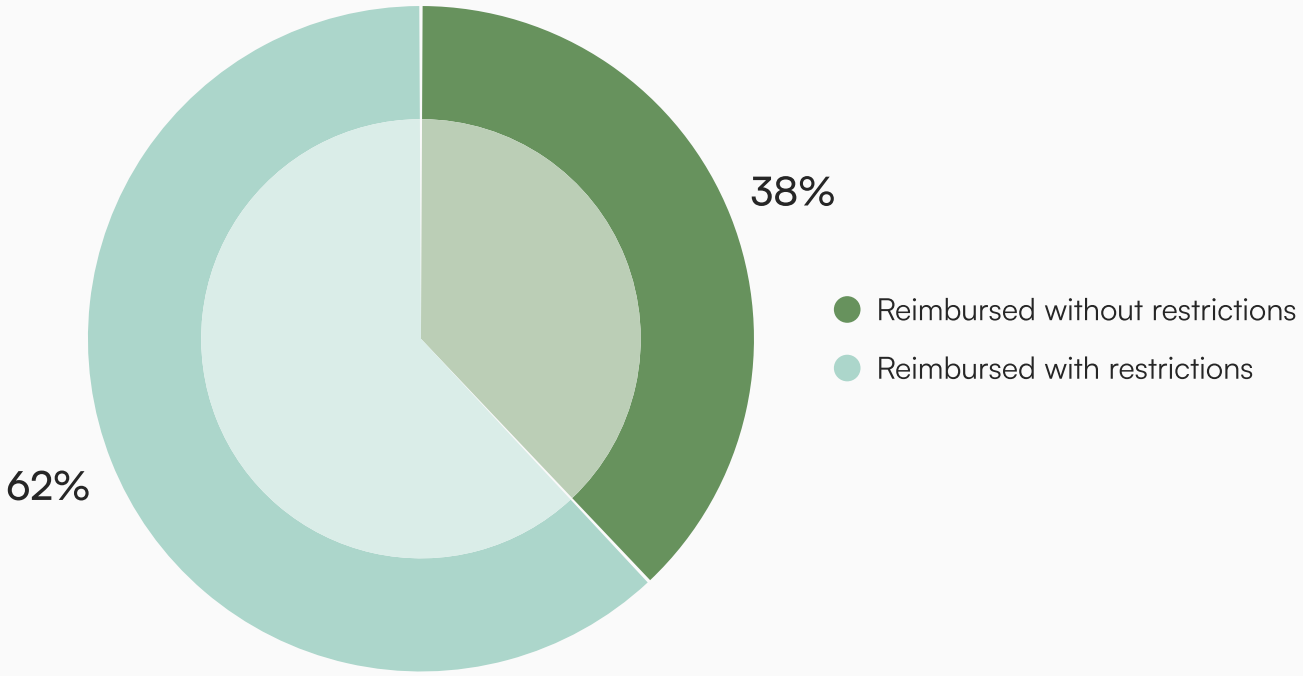


Absolute values



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Analysis of OMPs reimbursed by type of resolution

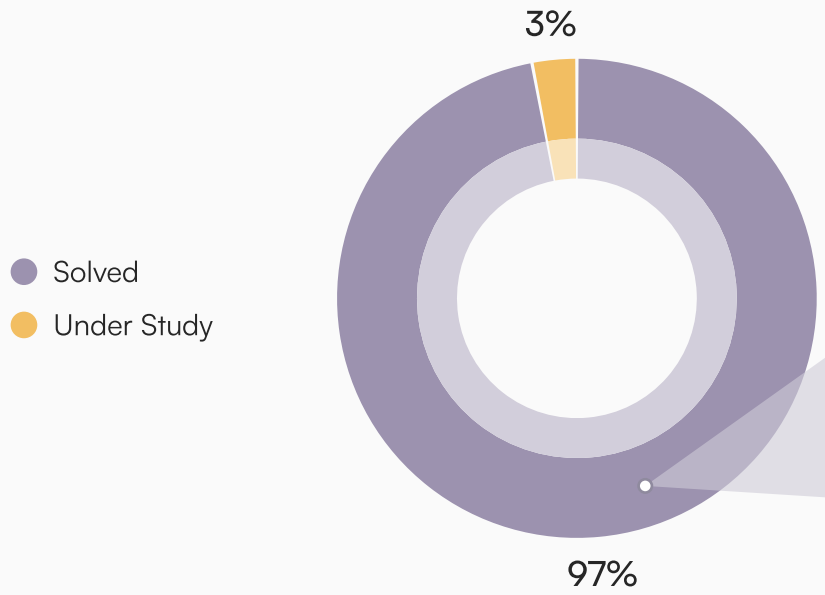


Of the **105 OMPs** included in the SNS, **62%** (65) are reimbursed **with restrictions**, either due to restrictions on the authorised indication(s), or because they have some indication not reimbursed in our country.

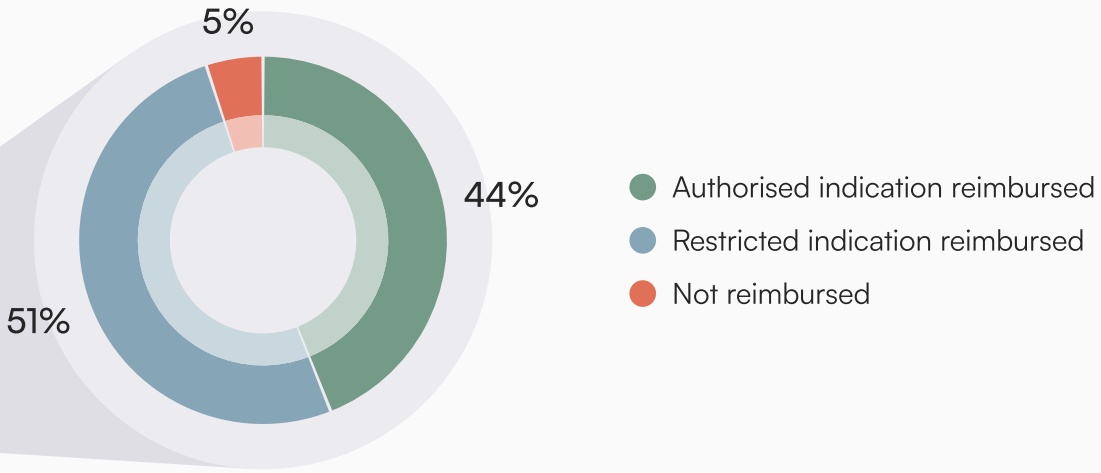
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Status of authorised indications of reimbursed OMPs

File status by indication



Resolution of the reimbursement file by indication

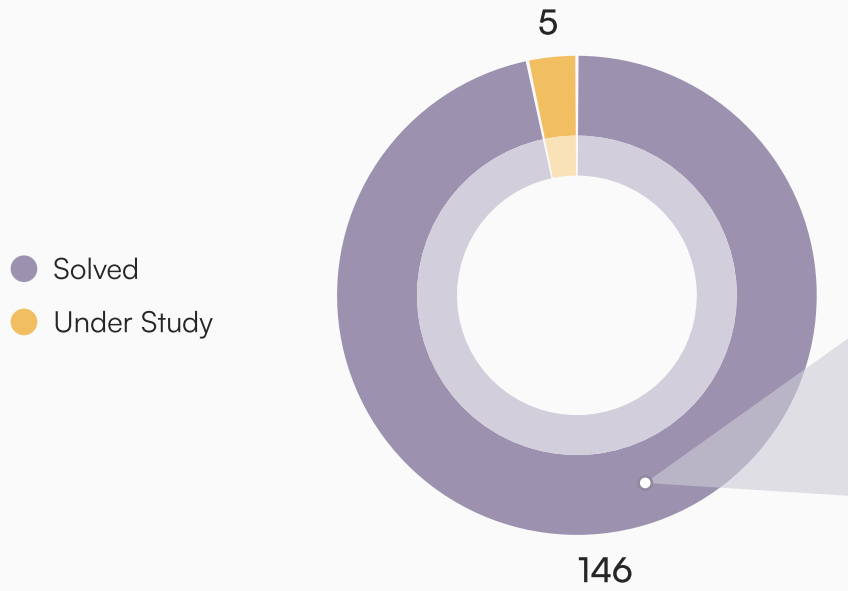


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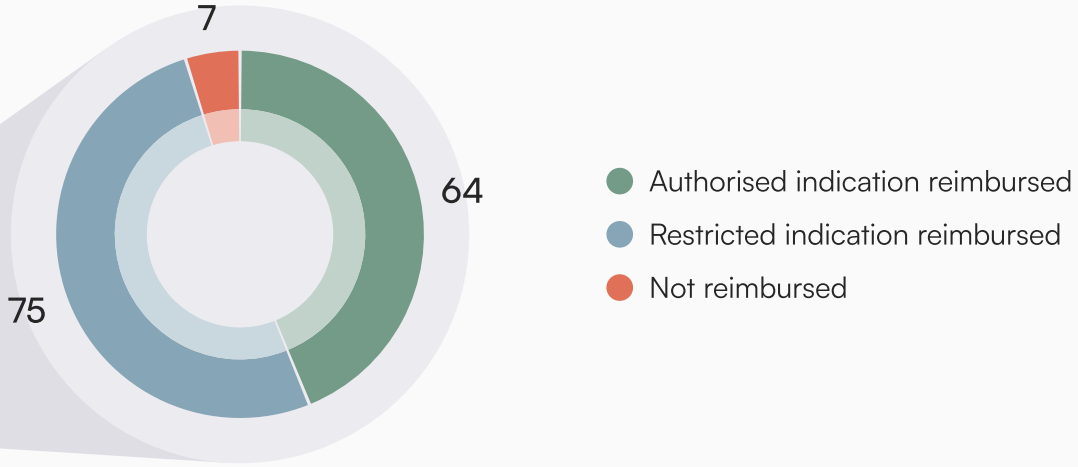
Source: BIFIMED (accessed in September 2026)

Status of authorised indications of reimbursed OMPs

File status by indication
Absolute values



Resolution of the reimbursement file by indication
Absolute values



Of the **151 indications authorised by the EMA,** 92% (139) are reimbursed.

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Orphan Medicinal Products

reimbursed by the SNS
between May and August 2026



New orphan medicinal products reimbursed in the second four-month period of 2026*

In the second four-month period of 2026, **2 new orphan medicinal products** have been reimbursed in Spain.

2 months

Average time from MA to NC

52 months

Average time from NC to reimbursement

100% Reimbursed with conditions by the SNS

100% Reimbursed with restrictions

1 OMP

Reimbursed in the first year after obtaining NC

1 OMP

Has taken more than three years to be reimbursed after obtaining the NC

0 OMPs

Reimbursed in their first CIPM meeting

1 OMP

Has attended at least five CIPM meetings before being reimbursed

*Data accumulated until August 31, 2026.

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New orphan medicinal products reimbursed in the second four-month period of 2026*

OMPs reimbursed in 2026 by type of reimbursement condition

Reimbursement conditions	Number	%
Seguimed	2	100%
Spending cap	1	50%
Price/volume agreement	1	50%
Highly complex protocol	2	100%
Valtermed	1	50%

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.

Source: BIFIMED (accessed in September 2026) and CIPM minutes (last published minutes: June 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 August 31, 2026.

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Details of criteria and conditions of the 2 OMPs reimbursed by the SNS in the second four-month period of 2026

	Brand name	Company	NC date	Reimbursement date	Decision on the reimbursement application	Months from MA to NC	Months from NC to reimbursement	CIPM meetings	Reimbursement criteria[1]	Reimbursement conditions
1	**ALYFTREK**	Vertex Pharmaceuticals	13/08/2025	01/06/2026	Yes, for certain indications	1	10	28/04/2026 25/02/2026	a) and c)	• Seguimed • P/V agreement • Highly complex protocol • Valtermed
2	**MYALEPTA**	Chiesi	06/11/2018	01/08/2026	Yes, for certain indications	3	94	17/06/2026 25/11/2021 10/06/2021 17/06/2020 28/11/2019	a) and c)	• Seguimed • Highly complex protocol • Spending cap

Source: BIFIMED (accessed in September 2026) and CIPM minutes (last published minutes: June 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 August 31, 2026.

[1] Criteria for reimbursement:

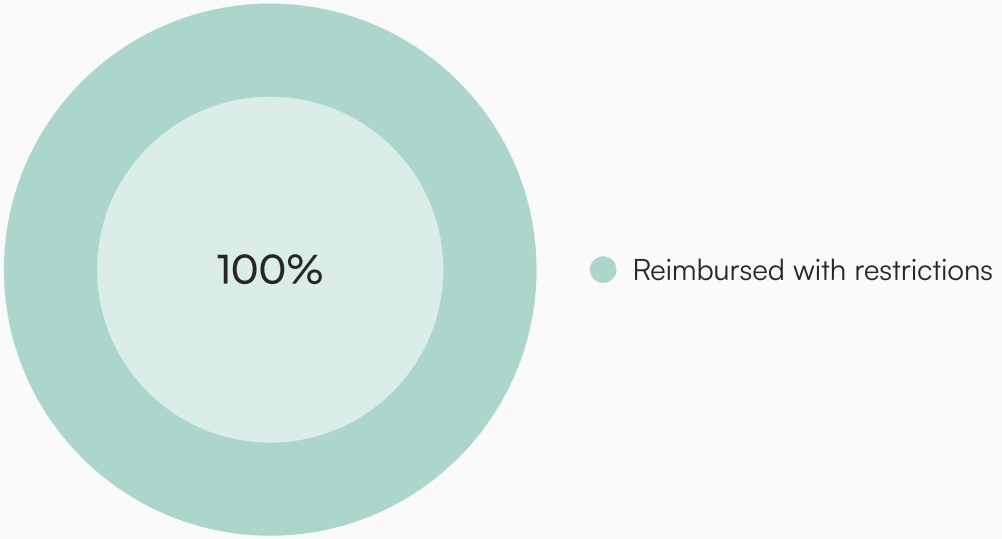
a) Severity, duration, and sequelae of the different pathologies for which they are indicated.

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

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Analysis of OMPs reimbursed in the second four-month period of 2026 by type of resolution

By type of resolution



Absolute values



The **2 orphan medicinal products reimbursed** in the second four-month period of 2026 were reimbursed with restrictions.

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Detail of indications of the 2 OMPs reimbursed

	Brand name	Company	Reimbursement situation	Decision on the reimbursement application	Reimbursed indications (Ministry of Health)	EMA indication
1	**ALYFTREK**	Vertex Pharmaceuticals	Yes, for certain indications	Yes, subject to the authorised indication	Alyftrek tablets are indicated for the treatment of cystic fibrosis (CF) in people aged 6 years and over with at least one non-Class I mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. Funding is restricted to the treatment of cystic fibrosis (CF) in people aged 6 years and over and weighing 40 kg or more with at least one non-Class I mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.	Alyftrek tablets are indicated for the treatment of cystic fibrosis (CF) in people aged 6 years and over who have at least one non-Class I mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.
2	**MYALEPTA**	Chiesi	Yes, for certain indications	Yes, subject to the authorised indication	Myalepta is indicated as a dietary supplement for use as replacement therapy to treat complications arising from leptin deficiency in patients with confirmed familial partial lipodystrophy or acquired partial lipodystrophy (Barraquer-Simons syndrome), in adults and children aged 12 years or over for whom standard treatments have failed to achieve adequate metabolic control. Reimbursement is restricted in the following situations: - It may be considered a treatment option in adult and pediatric patients (aged 12 years or over) with confirmed partial lipodystrophy (PL) and HbA1c greater than or equal to 8% and/or triglycerides greater than or equal to 500 mg/dl, in whom treatment of metabolic complications has failed. - It is not reimbursed for acquired partial lipodystrophy (Barraquer-Simons syndrome).	Myalepta is indicated as a dietary supplement and replacement therapy for the treatment of complications of leptin deficiency in patients with lipodystrophy (LD): - with confirmed congenital generalised LD (Berardinelli-Seip syndrome) or acquired generalised LD (Lawrence syndrome) in adults and children aged 2 years and over - with confirmed familial partial LD or acquired partial LD (Barraquer-Simons syndrome), in adults and children aged 12 years and over for whom standard treatments have failed to achieve adequate metabolic control.

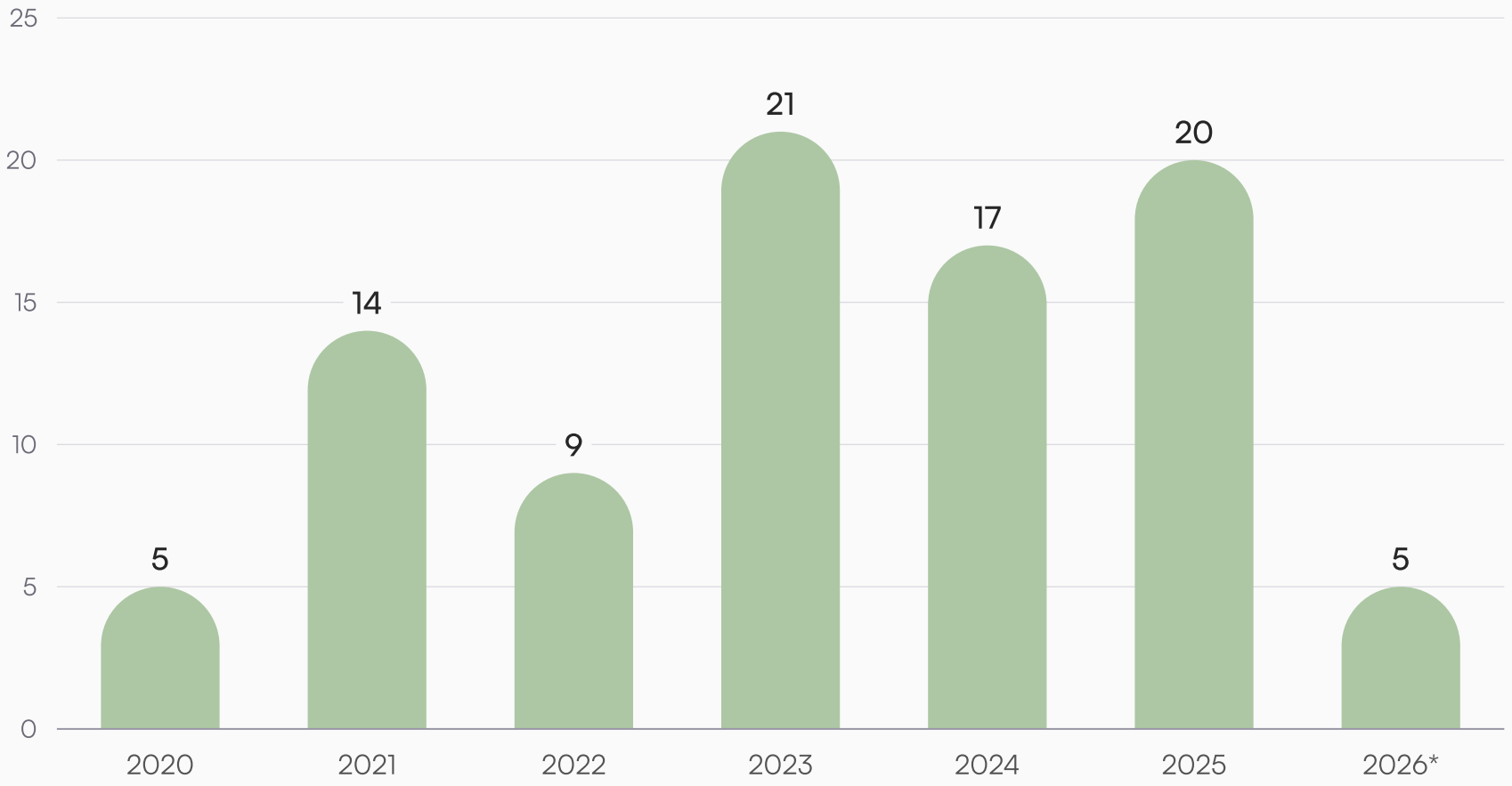
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Source: BIFIMED (accessed in September 2026)

Number of reimbursed OMPs in Spain 2020-2026*

ES

SNS

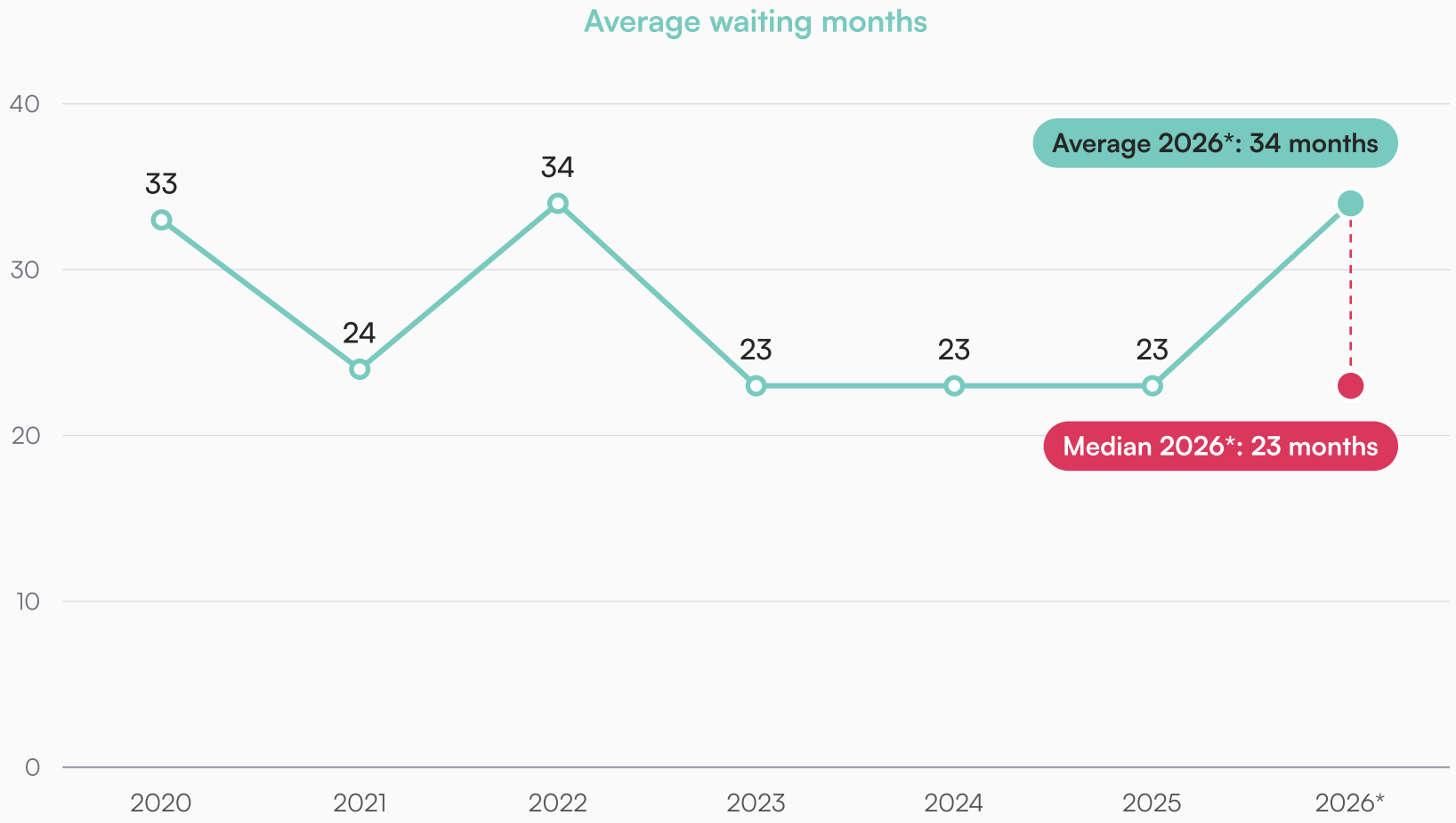


Note: The chart shows the annual situation of reimbursed medicines; however, the data are not cumulative year over year.

*Data accumulated until August 31, 2026.

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



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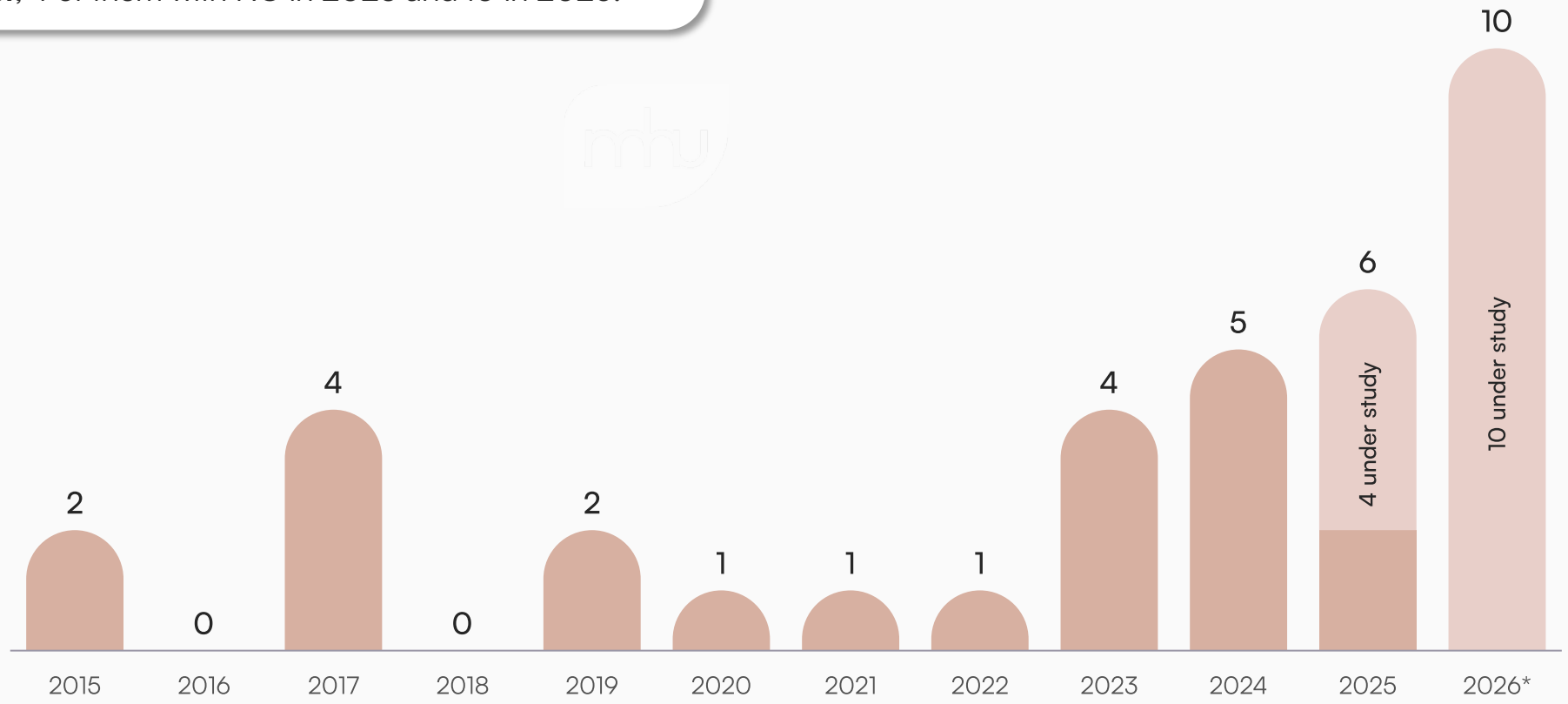
*Data accumulated until August 31, 2026.


Orphan
Medicinal
Products
not reimbursed

Orphan Medicinal Products not reimbursed in Spain by NC year

36 orphan medicinal products not reimbursed:

- **22 are not reimbursed by resolution.**
- **14 are under study or without reimbursement request**, 4 of them with NC in 2025 and 10 in 2026.

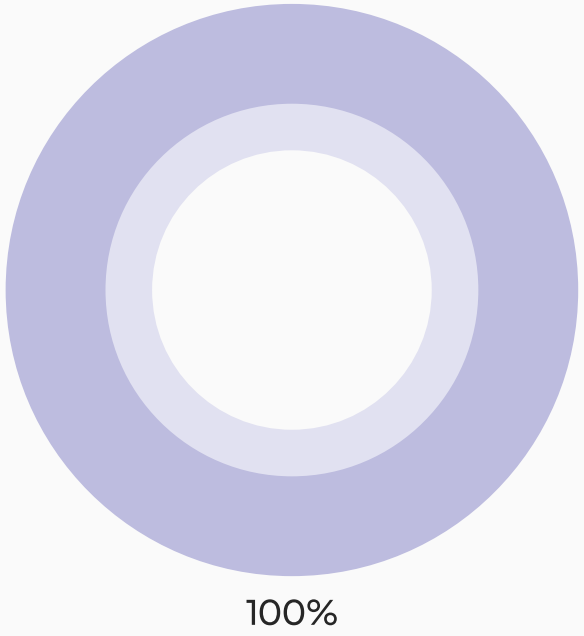


*Data accumulated until August 31, 2026.

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Orphan Medicinal Products with NC under reimbursement evaluation

Years of waiting for reimbursement



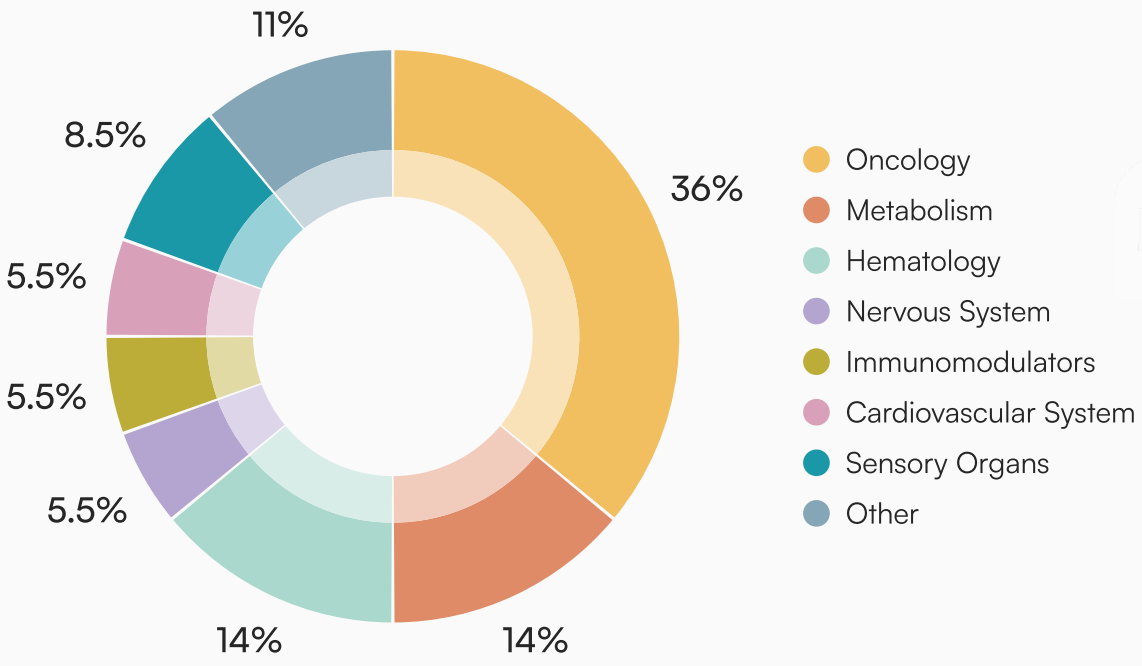
Absolute values



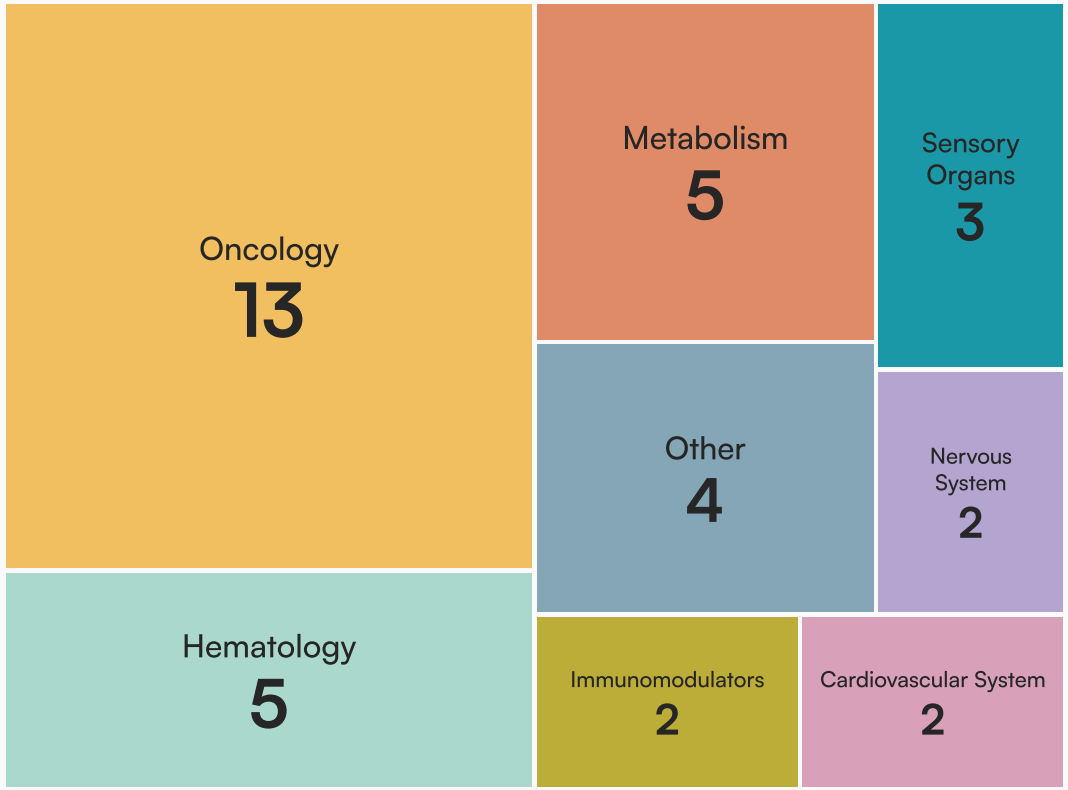
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OMPs with NC pending reimbursement

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	-	-	-	6
2	**IMDYLLTRA**	Oncology	Amgen	29/06/2026	-	-	-	2
3	**OGSIVEO**	Oncology	SpringWorks Therapeutics	02/01/2026	-	-	-	8
4	**REDEMPLO**	Cardiovascular System	Arrowhead Pharmaceuticals	07/07/2026	-	-	-	2
5	**REZUROCK**	Oncology	Sanofi	31/03/2026	-	-	-	5
6	**SEPHIENCE**	Metabolism	PTC Therapeutics	12/06/2026	-	-	-	3
7	**VORANIGO**	Oncology	Servier	02/01/2026	-	-	-	8
8	**WAYRILZ**	Hematology	Sanofi	17/04/2026	-	-	-	5
9	**ZEPZELCA**	Oncology	PharmaMar	05/06/2026	-	-	-	3
10	**ZYNYZ**	Oncology	Incyte	02/03/2026	-	-	-	6

*Data accumulated until August 31, 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	**ARTESUNATE**	Antiparasitic	Amivas	14/01/2025	24/09/2025 16/07/2025	01/11/2025	d) and e) d) and e)	20
2	**COAGADEX*** **	Hematology	BPL Bioproducts	31/03/2025	-	01/03/2026	-	17
3	**ENJAYMO**	Immunomodulators	Sanofi	29/12/2025	20/05/2026	-	d)	8
4	**ROMVIMZA**	Oncology	Deciphera Pharmaceuticals	26/11/2025	-	-	-	9
5	**TRYNGOLZA**	Cardiovascular System	SOBI	06/11/2025	-	-	-	10
6	**VYJUVEK****	Dermatology	Krystal Biotech	24/10/2025	17/06/2026	-	d)	10

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

**ATMP with orphan designation

*** COAGADEX has not been presented at any CIPM meeting. We have no public sources to ascertain the reasons for the non-reimbursement 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 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)	22
2	**CASGEVY****	Hematology	Vertex Pharmaceuticals	01/03/2024	19/11/2025 19/06/2025	01/01/2026	d) d)	30
3	**ELAHERE*****	Oncology	AbbVie	04/12/2024	-	01/03/2026	-	21
4	**VYLOY**	Oncology	Astellas	30/09/2024	28/01/2026 24/09/2025	01/03/2026	d) d) and e)	23
5	**ZTALMY*****	Nervous System	Marinus Pharmaceuticals	18/06/2024	-	01/10/2025	-	27

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

**ATMP with orphan designation

*** ELAHERE and ZTALMY have not been presented at any CIPM meeting. We have no public sources to ascertain the reasons for the non-reimbursement 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 Fabre	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)	44
2	**ELZONRIS**	Oncology	Stemline Therapeutics	20/10/2023	29/01/2025 17/07/2024	01/03/2025	c) and d)	35
3	**ROCTAVIAN****	Hematology	BioMarin	03/02/2023	19/06/2024	01/10/2024	c) and d)	44
4	**SOGROYA**	Hormone Preparations	Novo Nordisk	08/11/2023	24/04/2024	01/07/2024	d) and e)	34

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* 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.

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)	45

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* 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.

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)	69

* 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 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)	74

* 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)	82
2	**VERKAZIA**	Immunomodulators	Santen Pharmaceutical	08/04/2019	07/04/2021	01/07/2021	d) and e)	90

* 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 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**	Sensory Organs	Recordati	27/12/2017	05/05/2022 03/03/2022	01/12/2018	e) and d) e) and d)	106
2	**NINLARO**	Oncology	Takeda	06/02/2017	07/04/2021 03/02/2021	01/03/2019	d) d)	116
3	**OXERVATE**	Sensory Organs	Dompé	02/10/2017	-	01/07/2019	-	109
4	**XERMELO**	Metabolism	SERB	22/12/2017	-	01/06/2018	-	106

* 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****	Sensory Organs	Holostem	19/09/2015	-	01/08/2016	-	133
2	**STRENSIQ**	Metabolism	Alexion	15/10/2015	22/05/2024 20/03/2024	01/07/2018	d) d)	132

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

**ATMP with orphan designation. HOLOCLAR has not been presented at any CIPM meeting. We have no public sources to ascertain the reasons for the non-reimbursement decision.

[1] Criteria for non-reimbursement:

d) Rationalization of public expenditure 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

EU 73 ATMPs for rare diseases

- **55** ATMPs without MA
  - **46** included in the EMA PRIME program
  - **4** under evaluation by the CHMP
  - **5** academic
- **18** ATMPs with OD and MA*
  - **3** without NC
  - **15** with NC
    - **9** reimbursed by the SNS
    - **6** not reimbursed by the SNS
      - **5** with unfavourable resolution
      - **1** under study or without reimbursement request

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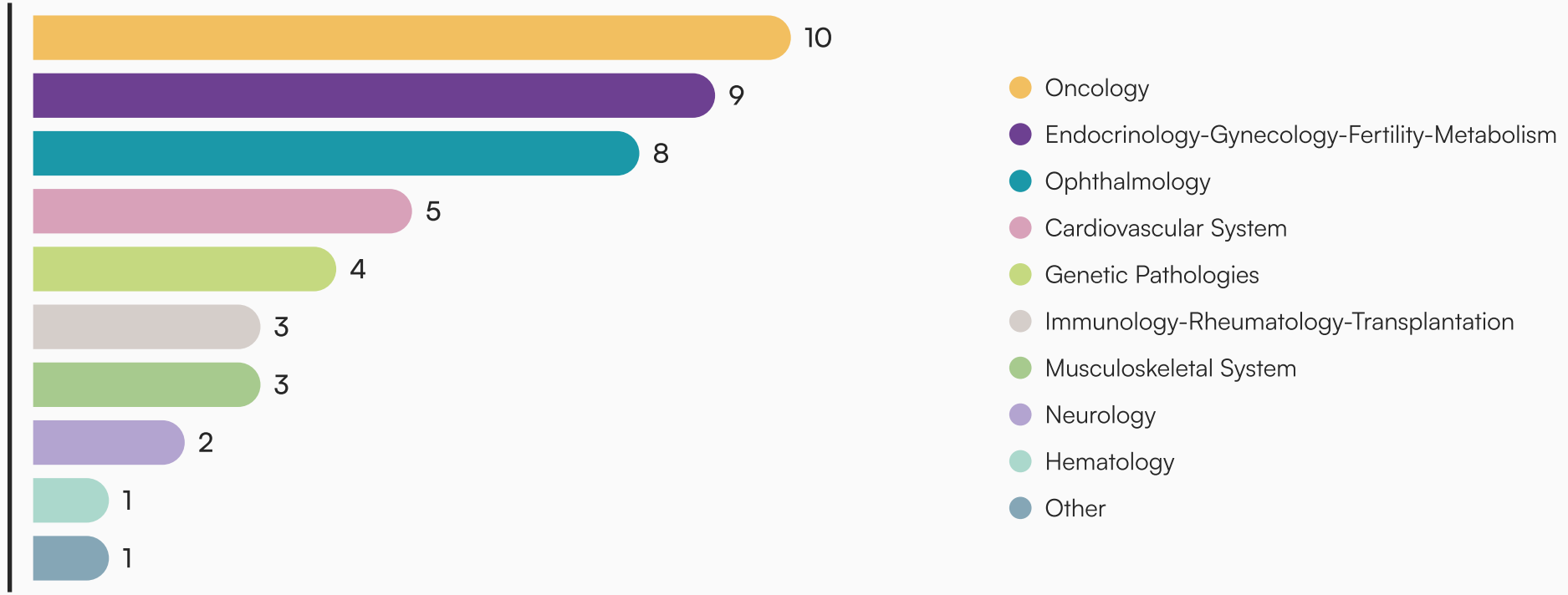
*The 18 advanced therapy medicinal products with OD and MA are included in the total of 231 OMPs with OD and in the 164 OMPs with MA, since they are ATMPs with a brand name.

ATMPs for rare diseases included in the EMA PRIME program

46

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 pharmaceutical companies so that **these therapies can reach patients sooner.**

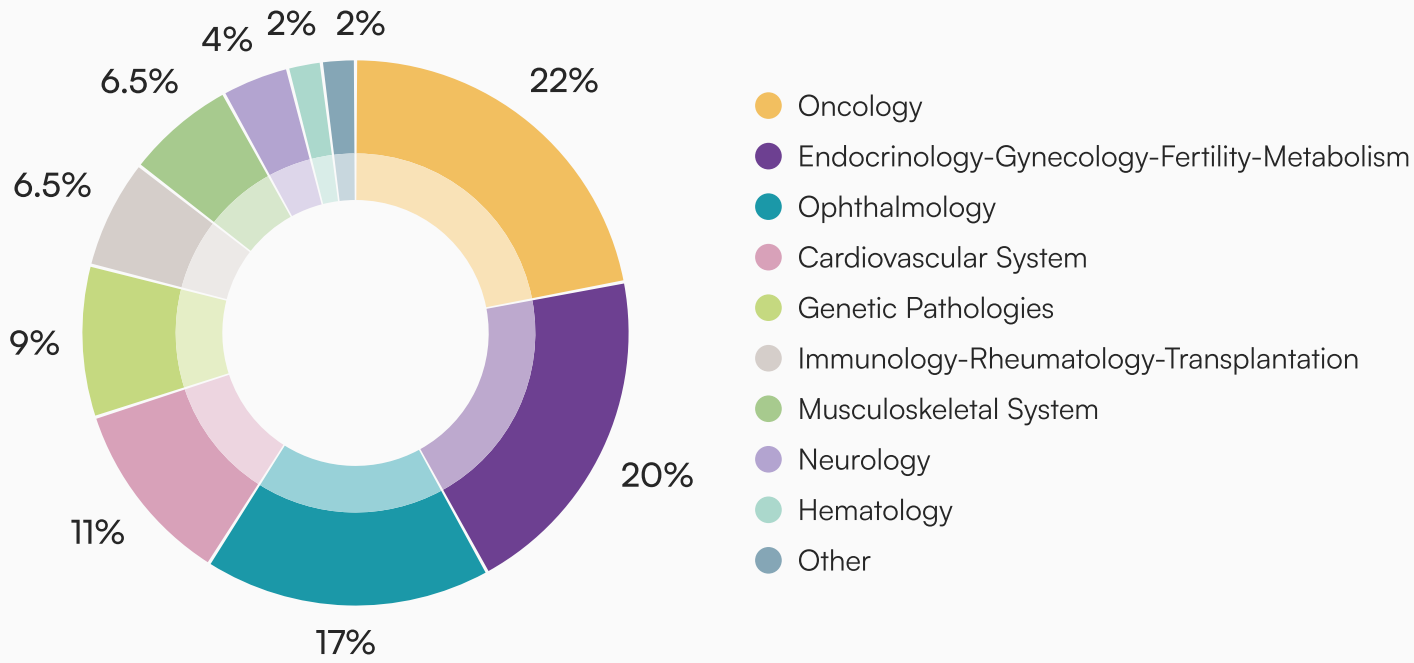
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ATMPs for rare diseases included in the EMA PRIME program

46

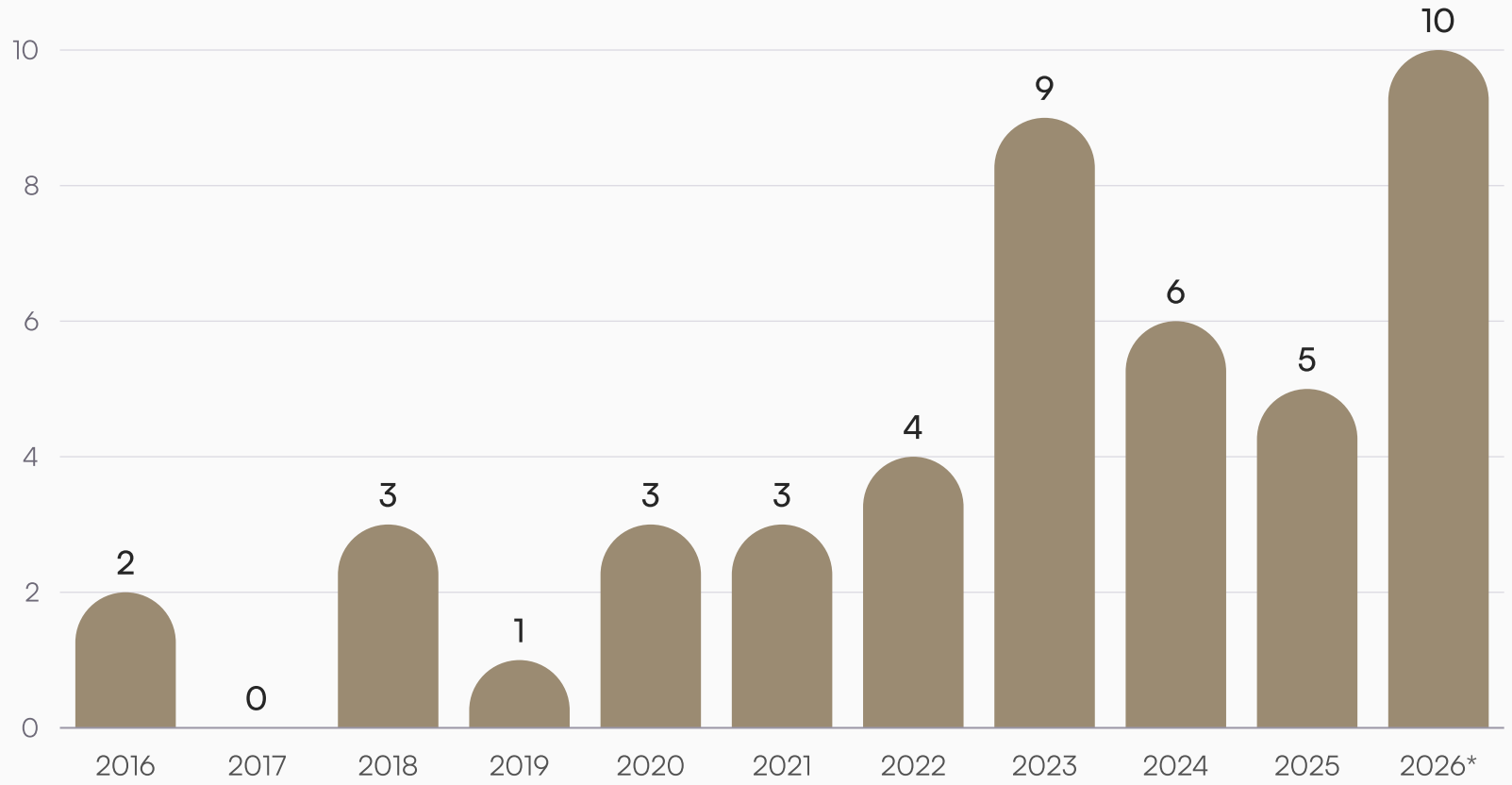
EU

Classification by therapeutic area



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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 August 31, 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	**Bilayer engineered skin composed of autologous keratinocytes and allogeneic fibroblasts embedded in an allogeneic plasma matrix**	Voisin Consulting	Dermatology	Treatment of adults and children with partial- and full-thickness dermal burns.	16/07/2026
2	**Lunsotogene parvec**	Regeneron	Sensory Organs	Treatment of hearing loss associated with biallelic variants of the OTOF gene in adults and children	21/05/2026
3	**Zamtocabtagene autoleucel**	Miltenyi Biomedicine	Oncology	Treatment of mature B-cell neoplasms	26/03/2026
4	**Zopapogene imadenovec**	FGK Representative Services GmbH	Respiratory System	Treatment of respiratory papillomatosis in adults	27/11/2025

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

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

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Details of academic ATMPs for rare diseases*

	Name	Therapeutic area	Authorisation 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**	Nervous System	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

*This slide shows the authorisations for the use of advanced therapy medicinal products granted by the Spanish Agency for Medicines and Health Products pursuant to Royal Decree 477/2014 of 13 June, which regulates the authorisation of non-industrially manufactured advanced therapy medicinal products.

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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*



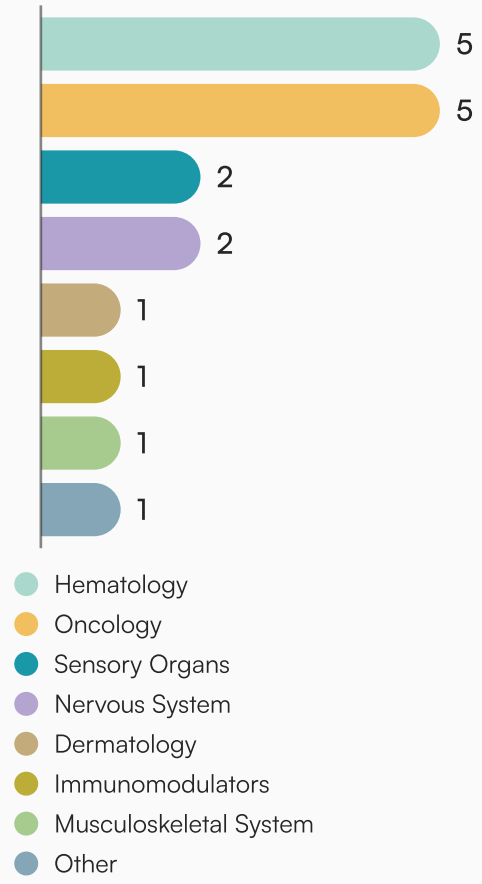
- Additional Follow-up

*The 18 advanced therapy medicinal products with OD and MA are included in the total of 231 OMPs with OD and in the 164 OMPs with MA, since they are ATMPs with a brand name.

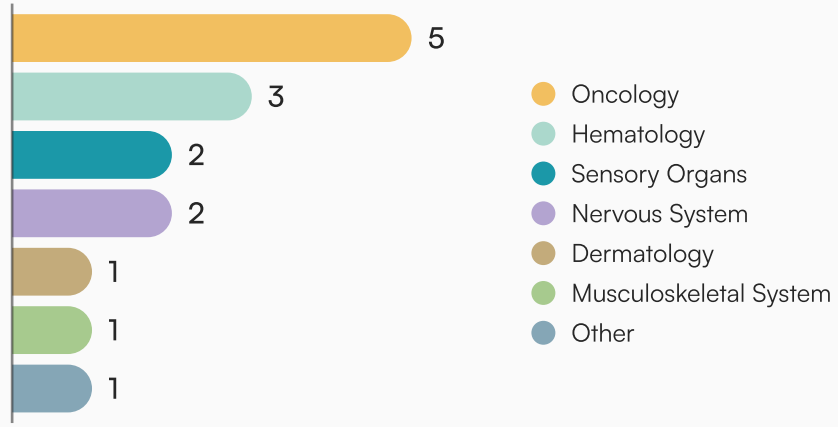
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Classification of the 18 ATMPs with OD and MA by therapeutic area

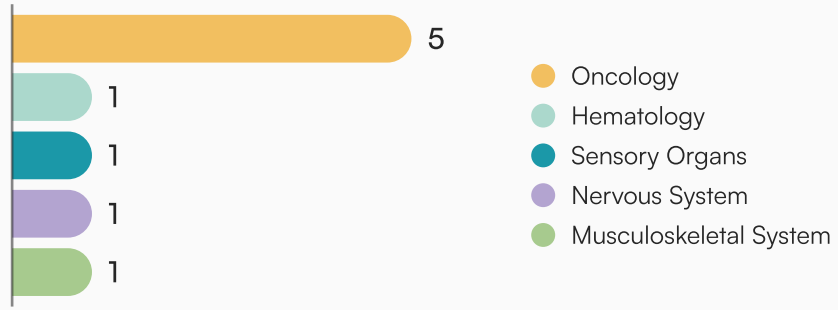
EU ATMPs with OD and MA by therapeutic area



ES ATMPs with OD and NC by therapeutic area



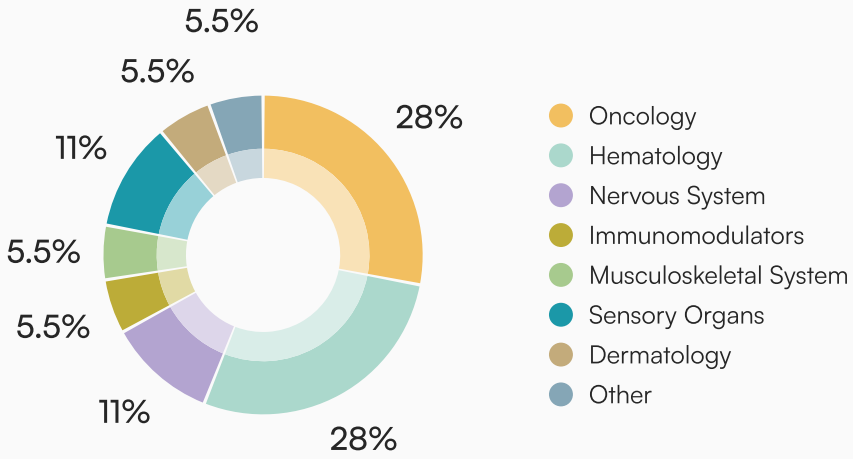
ES ATMPs with OD and reimbursed in Spain by therapeutic area



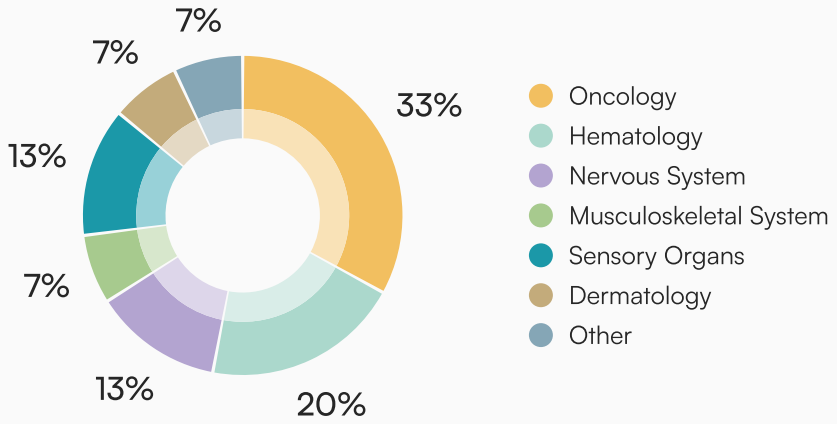
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Classification of the 18 ATMPs with OD and MA by therapeutic area

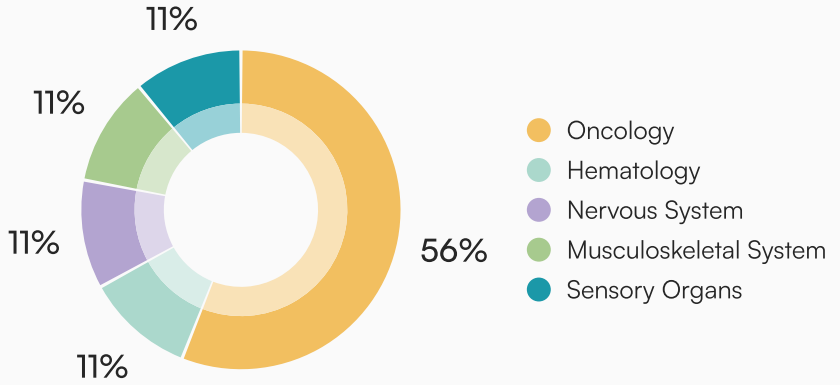
EU ATMPs with OD and MA by therapeutic area



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 refractory to 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 01/01/2026

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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 01/04/2025
5	**HEMGENIX**	Hematology	Treatment of severe and moderately severe hemophilia B in adult patients with no history of factor IX inhibitors.	CSL Behring	20/02/2023	15/03/2023	Reimbursed 01/11/2024
6	**HOLOCLAR**	Sensory 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 mm^2 of healthy limbus is required for the biopsy.	Holostem	17/02/2015	19/09/2015	Not reimbursed by resolution 01/08/2016

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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 arylsulfatase 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**	Sensory 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 01/10/2024
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.	Fondazione Telethon	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 aromatic L-amino acid decarboxylase (AADC) deficiency presenting with a severe phenotype.	PTC Therapeutics	18/07/2022	22/12/2022	Not reimbursed by resolution 01/01/2024
14	**VYJUVEK**	Dermatology	Treatment of wounds in patients with dystrophic epidermolysis bullosa (DEB) with mutations in the collagen type VII alpha 1 chain (COL7A1) gene.	Krystal Biotech	23/04/2025	24/10/2025	Under study or without reimbursement request
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	-

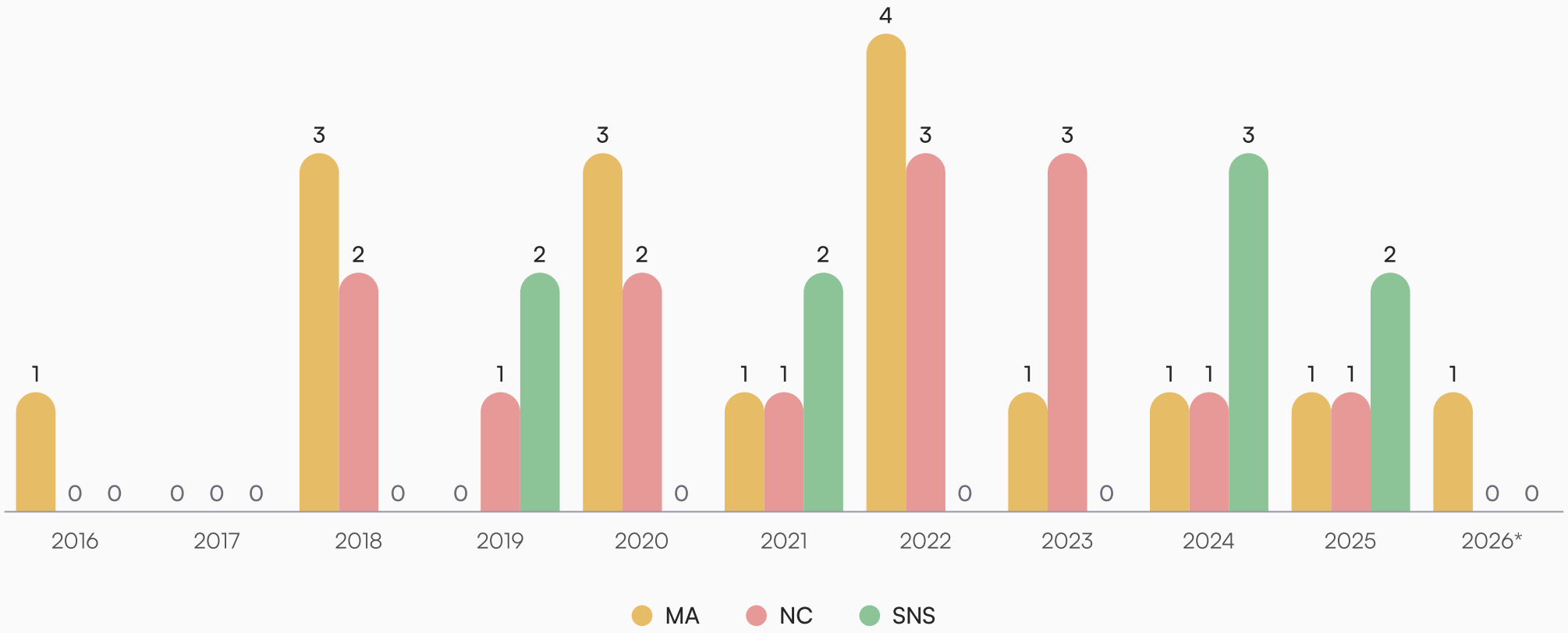
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Detail of the 18 ATMPs with OD and MA (6/6)

	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 included in this slide have a valid OD.

*Data accumulated until August 31, 2026.

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Reimbursed Advanced Therapy Medicinal Products

As of August 31, 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 included 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 NC 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 Behring	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 registry • 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

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

Source: BIFIMED (accessed in September 2026) and CIPM minutes (last published minutes: July 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] 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.
Article 96.2 of the Law on Guarantees and Rational Use of Medicines and Medical Devices

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Detail of the 9 reimbursed ATMPs (2/2)

	Brand name	Therapeutic area	Company	Reimbursement date	Months from NC to reimbursement	CIPM meetings	Reimbursement criteria[1]	Reported reimbursement price	Reimbursement conditions
6	**LUXTURNA**	Sensory 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 included in this slide have a valid OD.

Source: BIFIMED (accessed in September 2026) and CIPM minutes (last published minutes: July 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] 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.
Article 96.2 of the Law on Guarantees and Rational Use of Medicines and Medical Devices

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Detail of ATMPs with unfavourable reimbursement resolution

	Brand name	Therapeutic area	Company	Unfavourable 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**	Sensory 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 included in this slide have a valid OD.
Source: BIFIMED (accessed in September 2026) and CIPM minutes (last published minutes: June 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] 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.

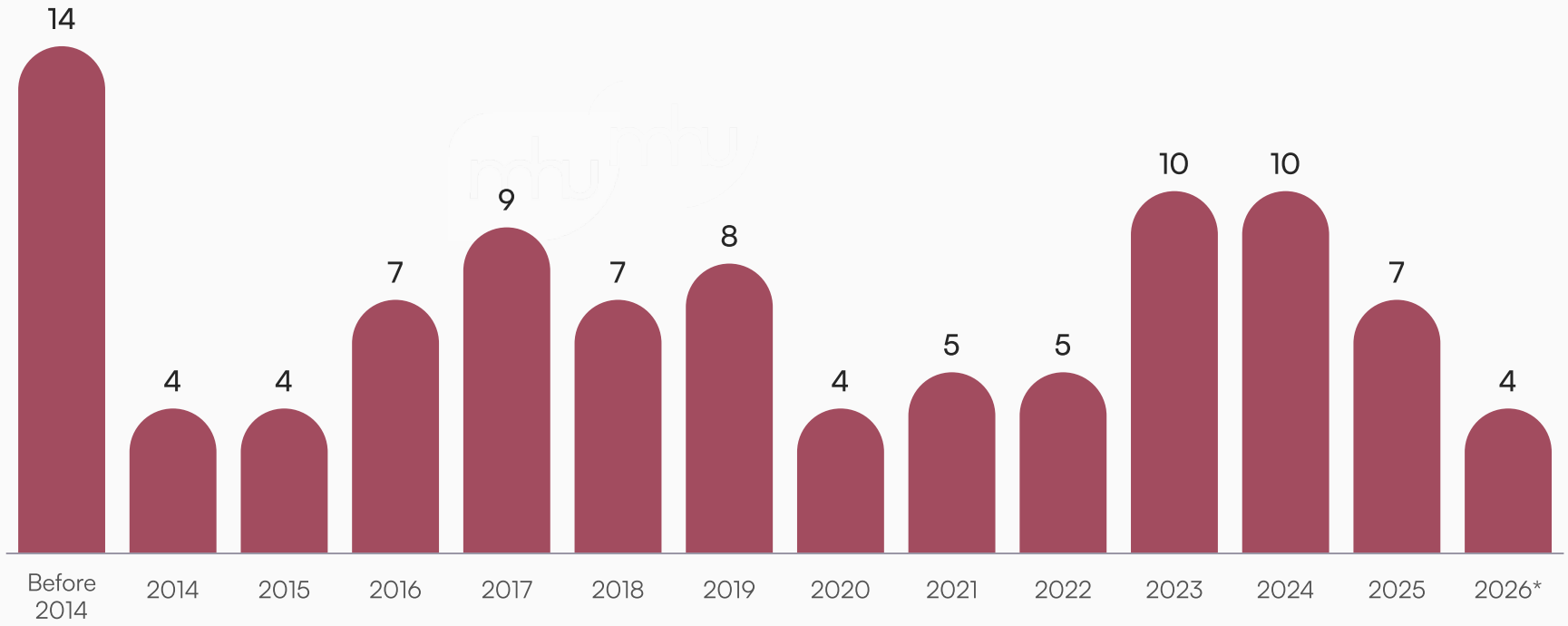
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Medicinal products indicated for rare diseases
that have lost orphan designation

Reimbursed medicinal products for rare diseases that have lost OD

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

Number of reimbursed medicinal products for rare diseases by the year of loss of OD



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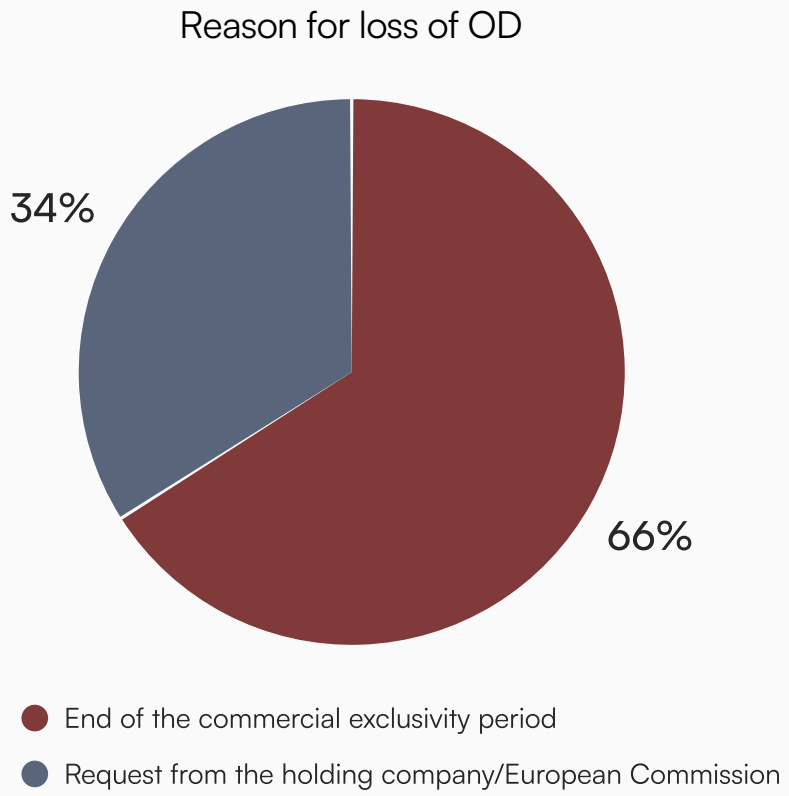
Source: Medicines that have lost orphan designation between 2008 and 2020 were obtained from the Orphanet analysis "List of medicinal products for rare diseases in Europe, August 2021". This list has been completed with medicines that lost orphan designation in 2021, 2022, 2023, 2024, 2025, and 2026.

*Data accumulated until August 31, 2026.

Reimbursed medicinal products for rare diseases that have lost OD

Of the **98** medicines indicated for rare diseases that have lost orphan designation but are still reimbursed by the SNS:

- **34%** (33) lost orphan designation **at the request of the marketing authorisation holder or by a decision of the European Commission.**
- **66%** (65) was due to the **expiration of the established period of commercial exclusivity.**



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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.



Summary:

Key facts

01 During the second four months of 2026:

- **6 new medicinal products with OD** and brand name.
- **6 orphan medicinal products** have obtained marketing authorisation in the EU.
- **4 orphan medicinal products** have obtained a National Code.
- **2 orphan medicinal products** have been reimbursed by Spain's National Health System.

02 As of August 31, 2026, there is a total of:

- **231** medicines with OD and brand name.
- **164** orphan medicinal products with European marketing authorisation.
- **141** orphan medicinal products with a National Code in Spain, 86% of those with MA.
- **105** orphan medicinal products reimbursed by the SNS, representing 64% of products with MA and 74% of those with NC.

03 Orphan Medicinal Products approval conditions:

- **44 (27%) of the orphan medicinal products** authorised up to August 31, 2026, have been approved conditionally or under exceptional circumstances, while the rest correspond to standard approvals.
- More than half of the OMPs authorised in Europe and/or Spain are subject to additional monitoring: 105 (64%) in the case of those with MA, 86 (61%) of those with NC, and 60 (57%) of those reimbursed in Spain.
- Of the 18 orphan medicinal products with conditional approval and NC, **14 have been reimbursed (78%),** 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, **14% of the medicines with MA have not yet arrived in Spain** (23) and, of these, 61% received MA more than a year ago.

05 Orphan Medicinal Products reimbursed between May and August 2026:

- **2 products have obtained reimbursement** between May 1 and August 31, 2026.
- The **average waiting time** from obtaining the National Code to reimbursement by the SNS has been **52 months.**
- **So far in 2026** (5 medicines reimbursed), the **average waiting time** from obtaining the National Code to reimbursement by the SNS has been **34 months**, with a **median of 23 months**.
- **One medicine** has been reimbursed within the first year of obtaining the National Code, and **no medicine** has managed to secure reimbursement at its first CIPM meeting.
- **One medicine** took more than three years to be reimbursed after obtaining the National Code, having to go through 5 CIPM meetings.
- **100%** of medicines reimbursed during the second four-month period of 2026 have **conditional reimbursement**, and 100% have **restricted reimbursement** (based on the indications approved by the EMA).

06 Non-reimbursed treatments:

- **36** orphan medicinal products with NC **are not reimbursed**, either because:
  - they are under study or without a reimbursement request (14); of these, **100%** have been waiting **less than a year** for a favourable reimbursement resolution;
  - or because they have been excluded by resolution (22).

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07 Advanced Therapy Medicinal Products for rare diseases:

- As at 31 August 2026, **75%** of the **73 ATMPs for rare diseases do not have a marketing authorisation** (MA). Of these, 46 are included in the EMA's PRIME programme, 4 are currently under evaluation by the CHMP and 5 are academic ATMPs.
- **18 ATMPs have orphan designation and a marketing authorisation**, of which 28% are indicated for oncology and 28% are haematological agents.
- Of these 18 advanced therapy medicinal products, **15 already have a National Code** (NC). By therapeutic area, 33% are for oncology and 20% are haematological agents.
- **Nine advanced therapy medicinal products are reimbursed by the National Health System (SNS)**, and the majority of these are for oncological indications (56%). Furthermore, 100% of these medicinal products are subject to monitoring via the SEGUIMED procedure, and 89% are subject to a pharmacoclinical protocol via VALTERMED.
- Of the **six advanced therapy medicinal products excluded** from SNS reimbursement:

  - **5** have had reimbursement applications rejected between 2016 and the second four-month period of 2026.
  - **1** is under consideration or has not submitted a reimbursement application.

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

- **98 medicines indicated for rare diseases** have lost orphan designation but are still reimbursed by the SNS.
- Of those medicinal products:
  - **34%** have lost the OD at the request of the marketing authorisation holder or by a decision of the European Commission.
  - **66%** 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 of the Interministerial Commission on Medicine Prices (CIPM)

06 PRIME program: Priority Medicines of the European Medicines Agency

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Acronyms

- **AEMPS**: Spanish Agency for Medicines and Medical Devices
- **ATMPs**: Advanced Therapy Medicinal Products
- **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
- **COMP**: EMA's Committee for Orphan Medicinal Products
- **EMA**: European Medicines Agency
- **EU**: European Union
- **MA**: European marketing authorisation by centralised procedure
- **NC**: National Code
- **OD**: Orphan Designation
- **OMPs**: Orphan Medicinal Products
- **PRIME**: Priority Medicines program by EMA
- **SEGUIMED**: Monitoring of medicine supply in the national market of Spain
- **SNS**: Spain's National Health System
- **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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