



French **Rare Diseases** **Market** 2026 & Beyond

Ecosystem Challenges
&
Implications for Pharma Companies

The French rare diseases market offers growth opportunities, but capturing this potential requires pharma companies to go well beyond securing regulatory approval and reimbursement

Five key takeaways for pharma companies

1. Attractive growth

- A ~€ 6 B market today, potentially exceeding € 13 B by 2034

2. Access bottleneck

- Reimbursement delays remain long despite dedicated pathways

3. Patient identification

- Finding eligible patients is as critical as driving prescriptions

4. Post-launch evidence generation

- RWE, registries and PROs increasingly support access and reassessment

5. Ecosystem integration

- Expert centers, FSMRs, PAGs and databases must be embedded into the launch strategy



- Success requires an integrated strategy combining patient identification, market access, evidence generation and ecosystem engagement — from development through launch and lifecycle management

There are approximately 3 million patients in France suffering from rare diseases, most of which are of genetic origin

Key figures

- Approx. **3 M patients**, accounting for **~4%** of the **French population** suffer from rare diseases
- Most rare diseases start in childhood and are of genetic origin
- **10%** of deaths among children aged 1 to 5 years old are due to a rare disease
- Diagnosis can be quite **long** and **complex**...
- ... and **misdiagnosis** is common, meaning the actual number of patients may be higher

70% of rare diseases
start in childhood

72-80% of rare diseases
have a genetic origin

Average time to diagnosis:
4.7 years

- Approx. **7,000 rare diseases** identified...
- ... though patients are **concentrated** within a relatively small number of them
- To date, most rare diseases still have **no curative treatment**
- Only a small proportion of rare diseases (**9%**) lead to **loss of autonomy**...
- ... but about half impair motor, sensory, and/or cognitive abilities

450 rare diseases
account for 85% of patients

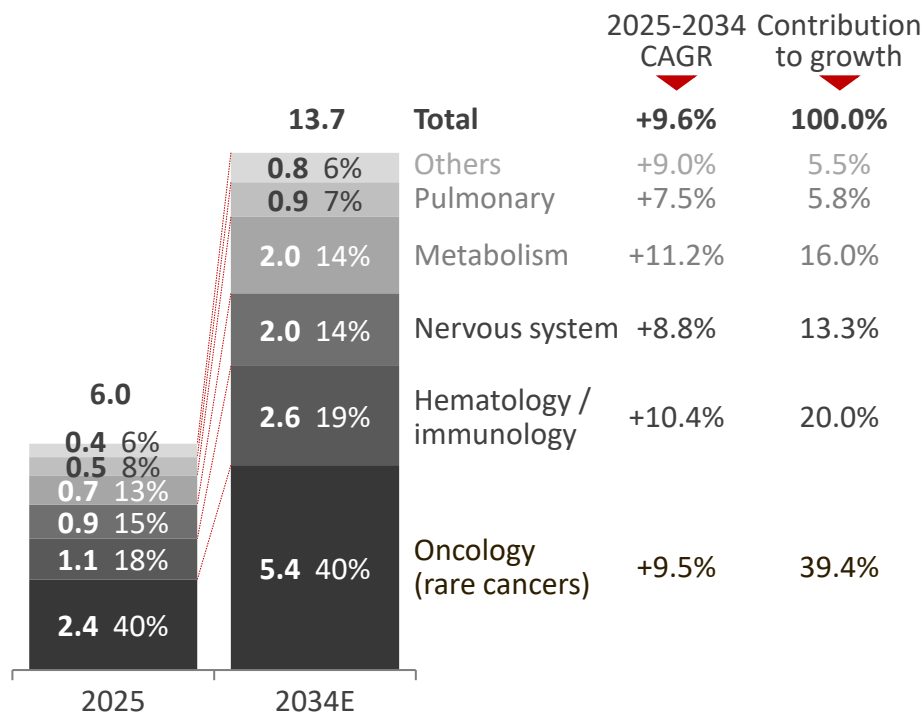
95% of rare diseases
have no cure

The French rare diseases market is estimated to grow at a **+9.6% CAGR** over the **2025-2034** period, driven by drugs for rare cancers, hemato/immunology diseases and metabolic disorders

Market sizing

French Rare Diseases market (€ B)¹

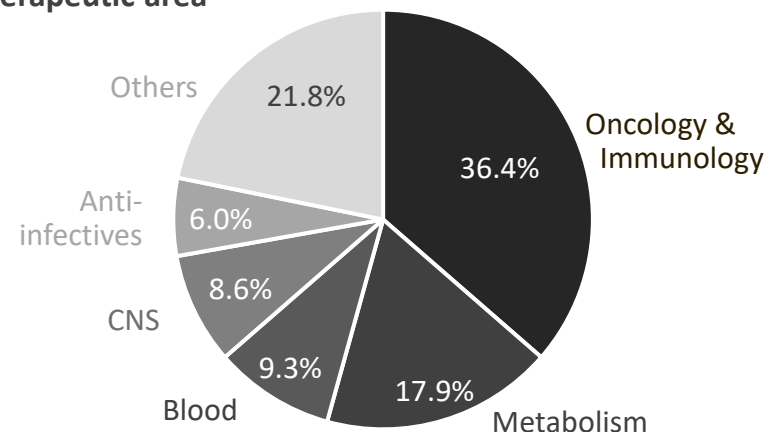
Rare diseases² represent approximately € 6 B, broken down by therapeutic area as follows:



Orphan drugs distribution in Europe

As a % of the total number of orphan drugs currently approved by the EMA

By therapeutic area



By therapy type

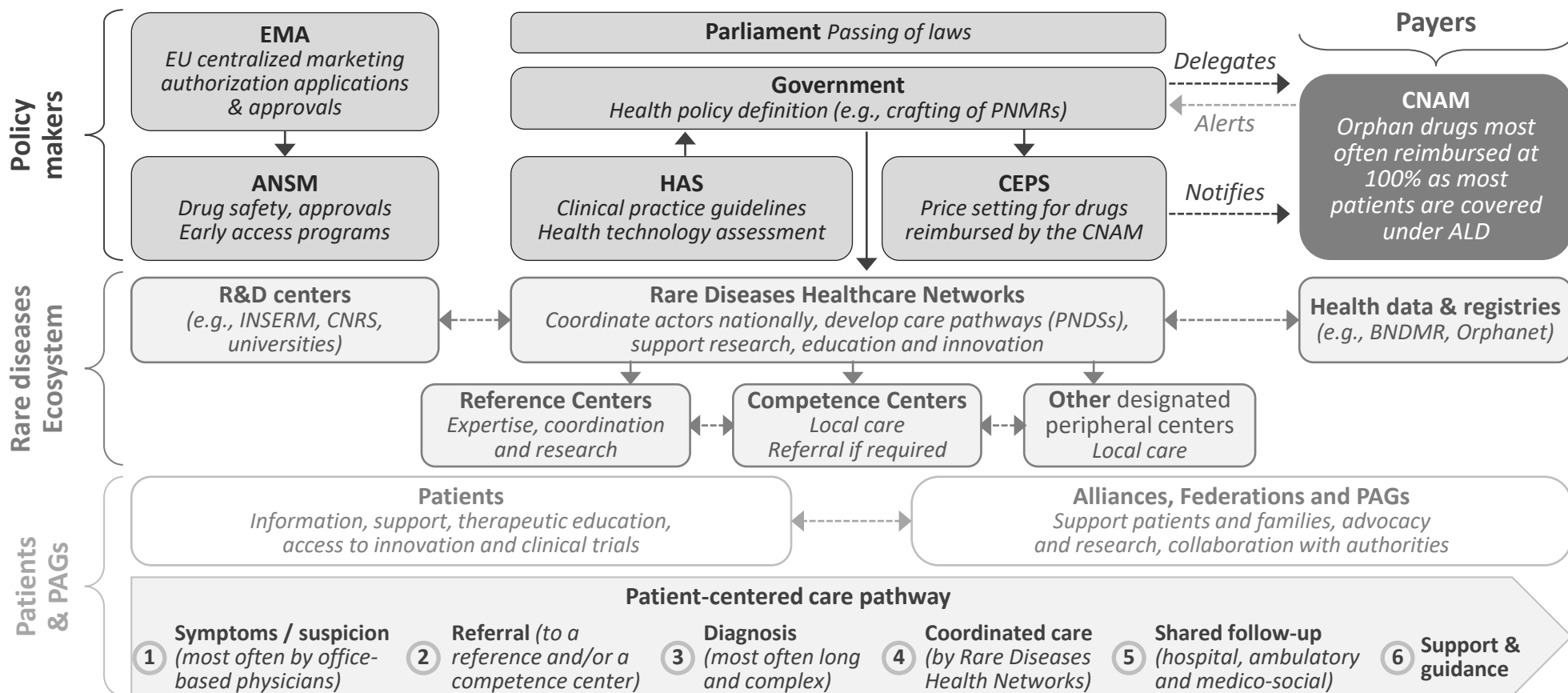


Sources: Medicinal products for rare diseases in Europe, Orphanet Report Series (Apr. 2025) – GERS database (Dec. 2025) – Smart Pharma Consulting analyses and estimates, based on EU and global market research reports, such as Vision Lifesciences (Feb. 2026) and Coherent Market Insights (Mar. 2026)

¹ Given the lack of precise market sizing data, estimates based on triangulation of GERS sales data, EMA/Orphanet product lists and international market growth benchmarks – ² The market definition includes orphan-designated and selected non-orphan medicines used predominantly in rare conditions

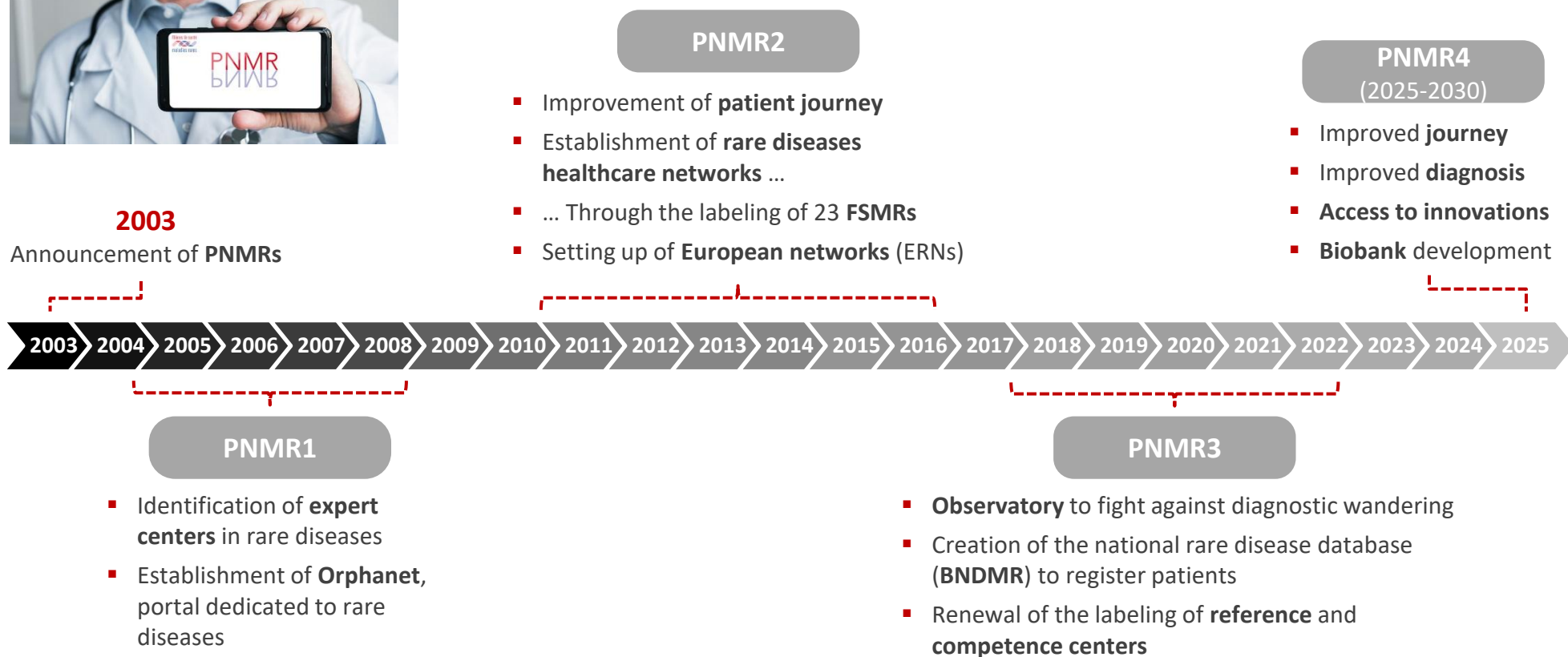
The rare diseases ecosystem is highly patient-centric, with dedicated pathways designed to shorten complex diagnoses and ensure coordinated care across specialized networks

Stakeholders' mapping



The rare diseases plans (PNMRs) introduced by French healthcare authorities aim to improve patients' diagnosis and management

Rare diseases plans (PNMRs)

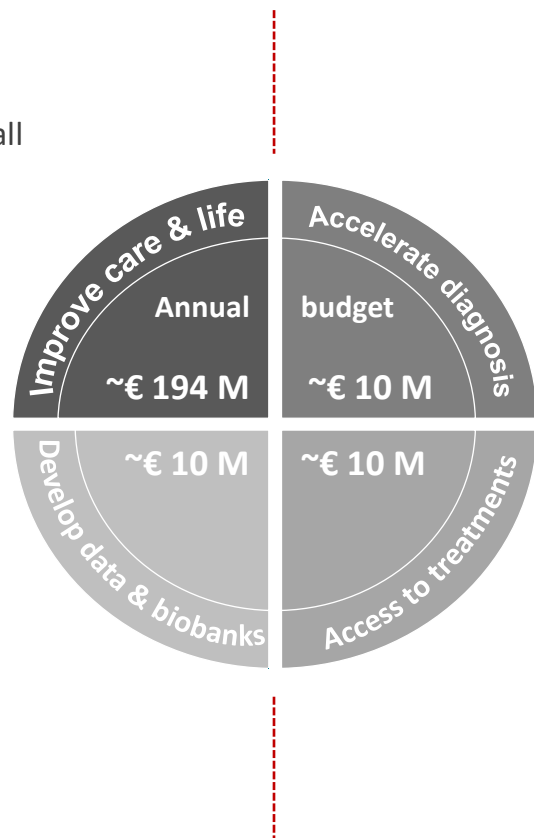


The 4th national plan for rare diseases (PNMR 4) aims to improve access to diagnosis, treatment and care, while strengthening research and European cooperation

Focus on the PNMR 4 (2025-2030)

- Strengthen the **network** of **reference** and **competence centers**
- Ensure **equitable access** to expertise across all regions (incl. overseas territories)
- Support **multidisciplinary** and **patient-centered** care

- Expand the **BNDMR**
- Promote **interoperability** of health data
- Foster:
 - **Clinical research**
 - **Real-world data** collection
 - **Biobank** accessibility



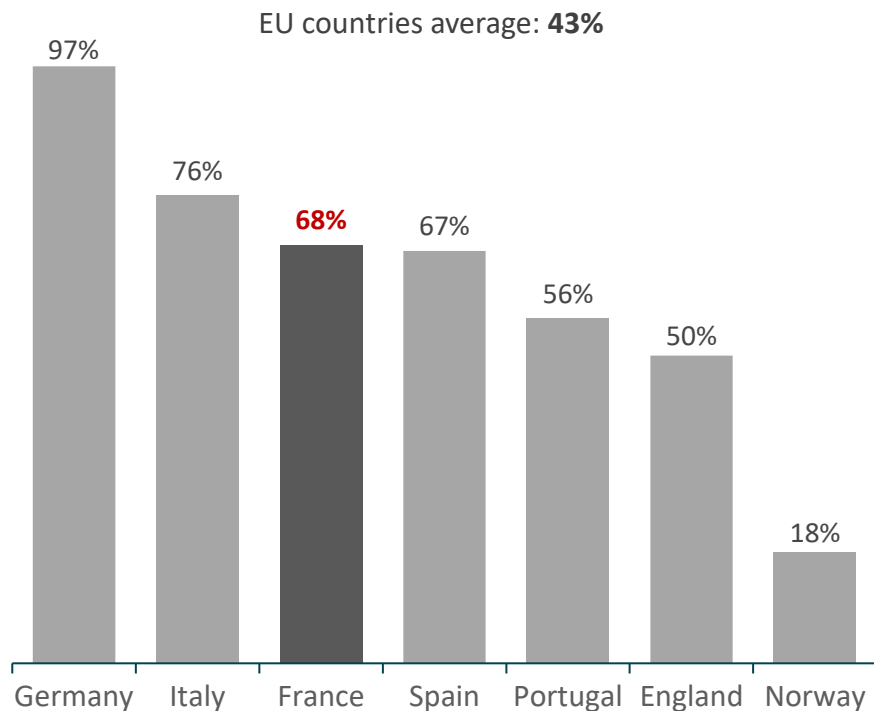
- **Shorten the diagnostic odyssey** through:
 - Genomics
 - Data sharing
 - Expanded newborn screening
- Promote:
 - **Early detection**
 - **Improved access** to specialized testing
- Encourage **R&D** of orphan drugs and advanced therapies
- Simplify **regulatory pathways**
- Improve **coordination** between **hospitals** and **pharma companies**
- Strengthen links with the **ERNs**

Availability of orphan drugs in France is much lower than in certain other EU countries
 due to strong access barriers and lengthier access timelines

Market access – Orphan drugs availability & time to market (2025)

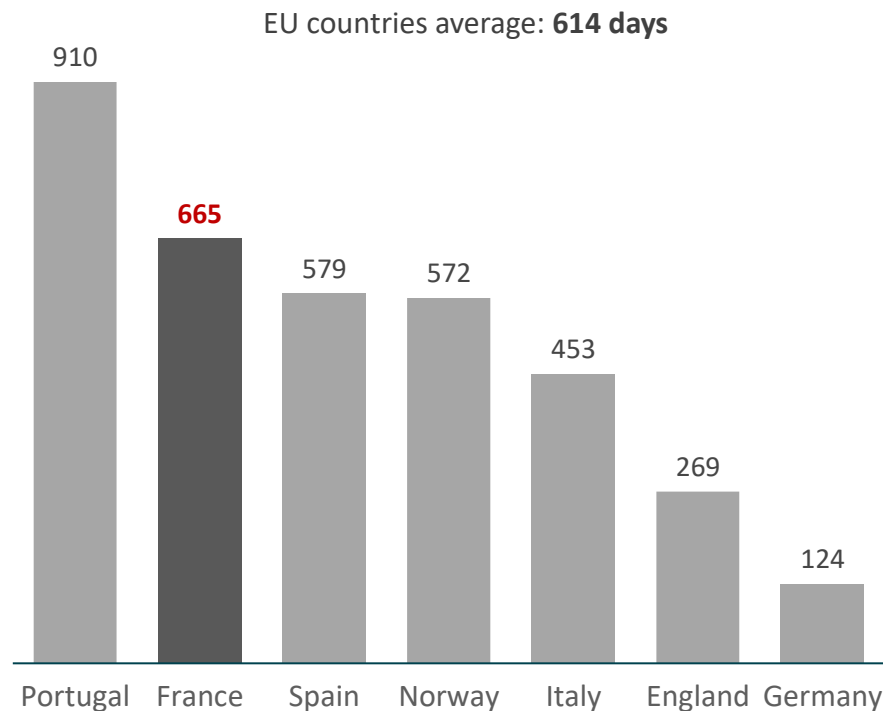
Availability

(% of new medicines available to patients¹)



Time to market access and reimbursement

(# of days between MA and reimbursement¹ in EU: **614 days**)



Orphan designation and then centralized Marketing Authorization (MA) are granted by the European Commission, while the ANSM and/or the HAS can authorize an early access

Market access – Marketing authorization



EMA

- Since 1999, the European Parliament has established the **COMP**, EMA's responsible **committee** for evaluating applications for **orphan designation**
- The COMP is required to assess within 90 days whether the **orphan designation criteria** are met:
 - The drug must **treat, prevent or diagnose life-threatening or chronically debilitating** conditions
 - The **prevalence** of the disease must be **≤ 1 in 2,000** in the EU or it is **unlikely** that marketing the drug would generate sufficient **returns** to justify the required **investment**
 - The drug **must be of significant benefit** to the patients
- Drugs designated as **orphan benefit from**:
 - **Protocol assistance**
 - **10-year marketing exclusivity**¹
- Marketing application is then submitted to the EMA's CHMP (Committee for Medicinal Products for Human Use)



ANSM & HAS

- In 2004, the European Parliament and Council determined that **all marketing authorizations** for **orphan medicines** in the EU will have to follow the **centralized authorization**
- However, although the centralized procedure grants authorization in all European Union members, there is **no obligation** to market the drug in every Member State
- Before the MA is granted, the ANSM issues an opinion on the presumptions of efficacy and safety of the product
- Since 2021, the HAS is responsible for granting or not an **Early Access Authorization (AAP)**, either before MA or after, but before routine reimbursement
- **Orphan drugs** may qualify for early access if there is **no other appropriate treatment available**, if the **treatment cannot be delayed** and the drug is presumed **innovative**

Sources: EMA website (March 2024) – Orphanet website (March 2024) – ANSM website (March 2024) – Smart Pharma Consulting analyses

COMP: Committee of Orphan Medicinal Products – EMA: European Medicines Agency – ANSM: National Agency for the Safety of Medicines and Health Products – HAS: National Authority for Health – AAP: Autorisation d'Accès Précoce – MA: Marketing Authorization
¹ In case of pediatric use, the marketing exclusivity may be extended to 12 years

Following centralized EU marketing authorization, orphan drugs should be evaluated by the Transparency Committee of the HAS before negotiating their price with the CEPS

Market access – Pricing and reimbursement (1/2)

Pricing & Reimbursement process

Marketing authorization (EMA)
(centralized for all orphan drugs)



Transparency Committee (CT)'s opinion



Clinical benefit
(SMR)



Reimbursement rate
(UNCAM)



Clinical added value
(ASMR)



Price setting
(CEPS)

Transparency Committee (CT)

- In France, orphan drugs benefit from an **accelerated procedure**, reduced from **90** to **30 days** after dossier submission
- **Comparative evidence** remains important but **methodological adaptations** are considered when justified by the disease or development context
- Regardless of the clinical added value (ASMR), orphan indications are covered by the **long-term illness provision** (ALD) which grants full reimbursement of treatment

Economic Committee on Healthcare Products (CEPS)

- The **2022 framework agreement** between the **CEPS** and the **LEEM** streamlined pricing negotiations for **orphan drugs**:
 - **Relevant pricing comparators**¹ clarification
 - Possibility to set a **capped budget**
 - **Price revision** if the number of patients evolves
 - **CEPS** authorization to be supported by **medical experts**

Sources: Transparency Committee website (March 2024) – CEPS website (March 2024) – National Health Insurance website (Mar. 2024) – Smart Pharma Consulting analyses

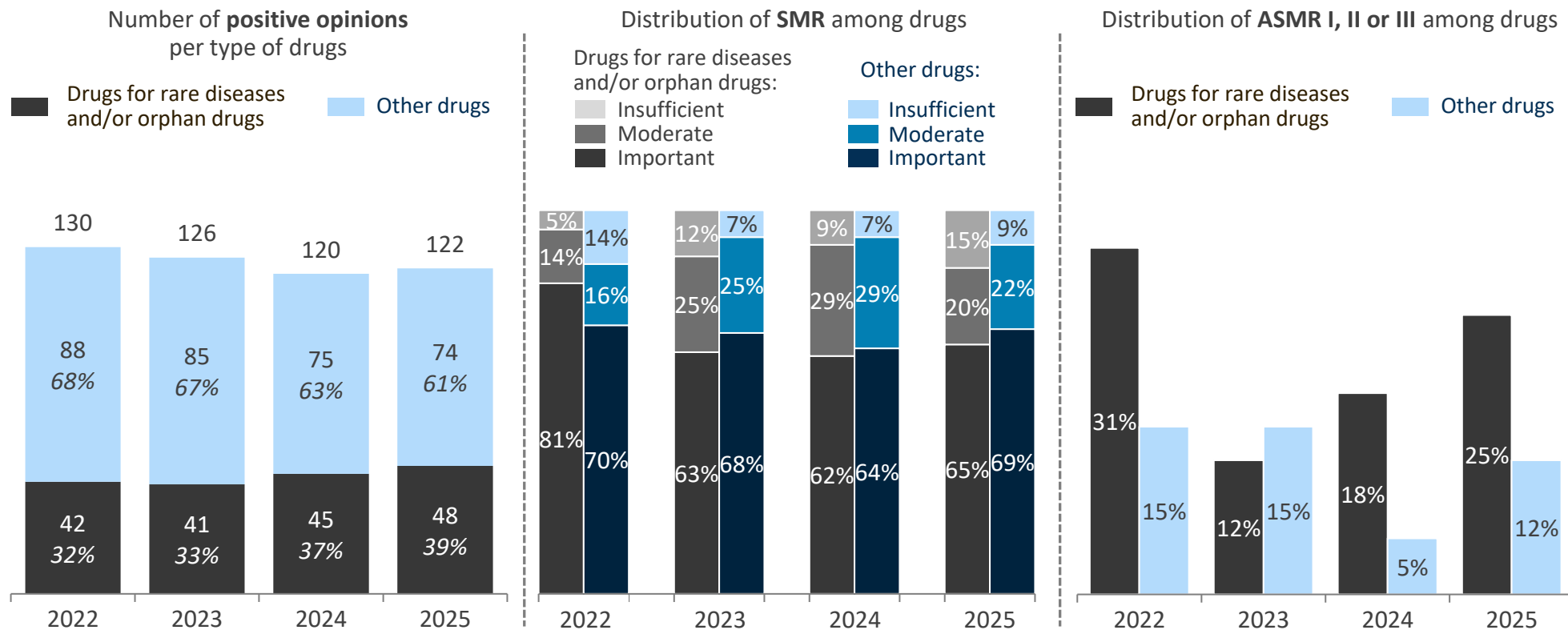
ALD: Long-term Condition – ANSM: National Agency for the Safety of Medicines and Health Products – CT: Transparency Committee – CEPS: Economic Committee on Healthcare Products – EMA: European Medicines Agency – LEEM: National Union of the pharmaceutical industry – UNCAM: National Union of Health Insurance Funds

¹ Same indication as the orphan drug and patent-protected

Drugs indicated for rare diseases and/or orphan drugs increasingly account for positive CT opinions and appear more likely to obtain ASMR I–III

Market access – Pricing and reimbursement (2/2)

Assessment of medicines by the CT of the HAS (2022 – 2025)



Sources: Rapports d'activité de la CT, HAS (2022 – 2025) –
 Smart Pharma Consulting analyses

CT: Transparency Committee – SMR: Clinical benefit – ASMR: Clinical Added Value

Early access programs require pharma companies to engage early with regulators, build strong evidence and anticipate pricing to ensure rapid uptake and sustainable access

Market access – Early Access Program process

- Introduced in 2021, the new framework of early access programs allows patients in therapeutic impasse to benefit from certain drugs that are not authorized for a specific therapeutic indication
- Five conditions must be met:
 - 1) The drug must be intended for **serious, rare or debilitating diseases**
 - 2) There is **no suitable treatment available**
 - 3) The treatment **cannot be deferred**
 - 4) The drug is presumed to be **innovative**, particularly with regards to a possible relevant comparator
 - 5) No **adverse opinion** from the EMA for drugs without marketing authorizations
- Once evaluated by the ANSM and the HAS, early access medicines are automatically covered by the National Health Insurance Fund

2 pathways

Early Access Authorization (AAP)

For drugs that will request a marketing authorization, or after MA but before reimbursement

Compassionate Access Authorization (AAC / CPC)

For drugs addressing unmet therapeutic needs, generally outside of a commercial pathway, incl. individual access to drugs and regulated off-label use

**2021-2025
Key figures**

140,000+ patients

With no appropriate treatment options were granted access to early access programs¹

80 days

Median processing time for initial applications (10 days less than the 90 days required by law)

17 months

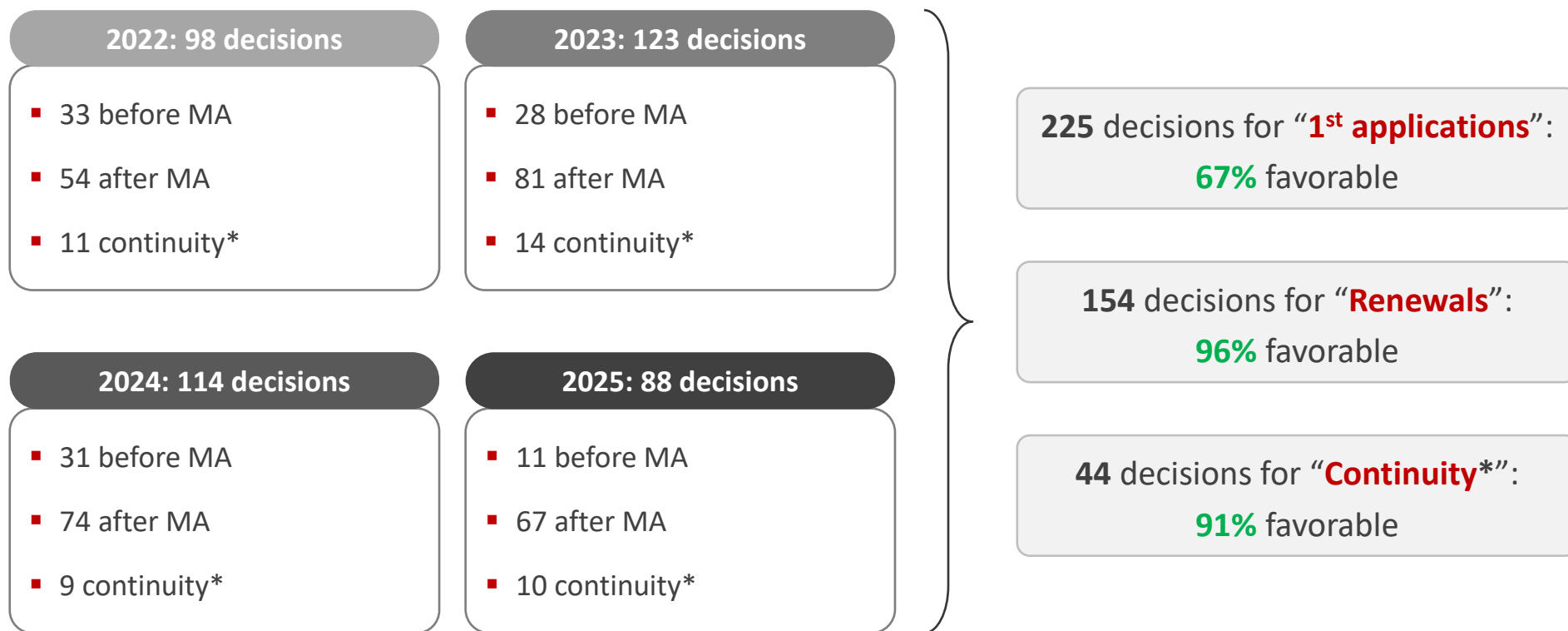
Before being reimbursed under common law

Sources: Decree n°2021-869 (Jun. 2021) – Bilan accès précoce, HAS (Nov. 2025) – Ministry of Health – Smart Pharma Consulting analyses

ANSM: National Agency for the Safety of Medicines and Health Products – AAC: Autorisation d'Accès Compassionnel (for individual use) – AAP: Autorisation d'Accès Précoce – CPC: Cadre de Prescription Compassionnelle (for off-label use) – HAS: National Health Authority – EMA: European Medicines Agency
¹ From Jul. 2021 to Jun. 2025

Over the last four years, the Transparency Committee of the HAS issued a total of 423 early access decisions, of which 67% favorable for first applications and 96% for renewals

Market access – Early Access Program decisions



Sources: Rapports d'activité de la CT, HAS (2022 – 2025) – Smart Pharma Consulting analyses

* For drugs that were previously included in an early access program prior to obtaining a marketing authorization, and for which continued inclusion in the program is requested after the marketing authorization has been granted

Rare diseases healthcare networks (FSMRs) are key partners for pharma companies seeking to engage with experts, understand patient pathways, support treatment development and launch

Rare diseases ecosystem – Healthcare networks

- The FSMRs are **national networks** created to **coordinate the actions** of rare diseases' stakeholders and structures
- They are organizations anchored **within a healthcare facility**, under a **medical coordinator**, and bringing together, for a given group of diseases:
 - Reference centers
 - Competence centers
 - Diagnostics and research labs
 - Educational, social and medico-social structures
 - Learned societies
 - Universities
 - Patients' associations
- **Two objectives:**
 - Promote a **better understanding** of the **care pathways**
 - **Coordinate efforts** among healthcare **facilities** and **stakeholders**
- **Four areas of action**

1

Improving care and patient pathways
 (e.g., prevention, diagnosis, care)


2

Fostering education, training and information
 (e.g., prevention, diagnosis)


3

Promoting and coordinating fundamental and clinical research


4

International coordination
 (e.g., ERNs)



23 networks provide structured entry points into highly fragmented disease populations, each comprising reference and competence centers

Rare diseases ecosystem – Reference and competence centers



- A common Rare Diseases website **filières de santé maladies rares** brings together all the information a patient, caregiver or healthcare professional could look for, including:
 - **Educational material** for patients and caregivers
 - **Information** for healthcare professionals **to minimize medical confusion** (e.g., a support tool to guide patients to the right specialists)
 - A list of **available training** courses for **expert patients** and **expert parents**
 - A presentation of the **rare diseases networks** and **centers**
 - A presentation of the **national database** of patients suffering from rare diseases (**BNDMR**)
 - Various tools to **facilitate the patient's life** (e.g., “childhood to adulthood” guide)

Several types of centers cover France to bring together specialized HCPs (medical, paramedical, psychological, medico-social, educational) to ensure a multidisciplinary care approach

Rare diseases ecosystem – Expert centers

Rare diseases reference centers (CRMR) (national level)

- **Highly specialized** expertise
 - **Complex diagnosis**
 - Definition of treatment and personalized care plan
 - Organization of multidisciplinary team meetings
 - **Clinical research**
 - Training & support to other structures
- CRMR: min. 75 patients
 - Coordination CMR: min. 150 patients
 - Labelled for 5 years
 - 387 CRMR (2023-2028 period)

Rare diseases competence centers (CCMR) (regional level)

- **Follow-up** and **continuity** of care
 - **Guidelines** and **protocols** implementation
 - **Stabilized** patients' management
 - **Telemedicine** activities
 - **Link** between **hospital** and **office-based HCPs**, **social** and **educative teams**
- CCMR: min. 25 patients and active user of the BNDMR
 - Labelled for 5 years
 - 1,708 CCMR in 124 healthcare facilities (2023-2028 period)

Resources & Competence centers (CRC) (local level)

- Functional support in specific aspects of care pathway: **rehabilitation**
 - **Education** and **training** to the patients and caregivers
 - **Diffusion** of clinical and functional **best practices**
 - **Patient orientation** towards the appropriate resources
- Only for cystic fibrosis, rare hemorrhagic diseases and spinal muscular atrophy (SMA)

Other healthcare & medico-social facilities (local level)

- **First medical contact** / Suspicion of a rare disease
- **Referral** to the appropriate **center**
- Management of **associated comorbidities**
- **Support** of the **care pathway**:
 - Rehabilitation activities
 - Education / Workplace adaptation
 - Home care services
 - Psychological support
 - Link to Patient Advocacy Groups
 - Etc.

National Protocols for Diagnosis and Care (PNDS) are important tools for understanding and optimizing rare disease patient pathways, from diagnosis to treatment and long-term follow-up

Rare diseases ecosystem – PNDS

An expert guideline

- National Protocols of Diagnosis and Treatment (**PNDS**) are **clinical practice guidelines developed** and **disseminated** by corresponding **CRMR** and **CCRM** ...
- ... with a **method** elaborated **by the HAS** (but without formal HAS validation)
- They are updated every 5 years
- Each PNDS includes **recommendations on**:
 - Preliminary **exploration** and **diagnosis**
 - **Care pathway** definition
 - Long-term **follow-up**
 - **Contacts to the PAGs**
 - **Contact to the coordination center, expert platforms and healthcare network**
 - A short **summary for attending GPs**

Method to elaborate a PNDS

- The method was developed by the HAS in 2012 to produce and update each PNDS
- The **key steps** are as follows:
 - Define the **scope** and **objective** (the rare disease the PNDS will address)
 - Set up a **multidisciplinary expert group**
 - **Review evidence** (e.g., diagnosis, treatment): systematic literature search and assessment of clinical evidence
 - **Build recommendations**: diagnostic pathway, treatment strategy, monitoring, complications management
 - **Write scientific justification**: separate document explaining evidence, reasoning and strength of recommendations
 - **Prepare summarized documents** for clinicians and attending GPs

BNDMR (National Rare Diseases Database) is a strategic resource for understanding rare disease populations, supporting clinical development and generating real-world evidence (RWE)



Rare diseases ecosystem – BNDMR

Presentation

- **National database** dedicated to rare diseases in France
- Initiated from 2012 onwards as **part of** the French National Plan for Rare Diseases (**PNMR**)
- Progressively **deployed** at national level **through expert reference centers**

Key data (2025)

- **+1.9 million patient records**
- **7,300 diseases** covered
- **Data collected from 154 hospitals** (rare diseases centers)
- **Pseudonymized data**

Objectives

- **Improve knowledge** of rare diseases
- Structure a national standardized **data warehouse**
- **Support research, epidemiology and therapeutic innovation**
- Inform / **guide public health policies** (e.g., PNMRs, PNDs)

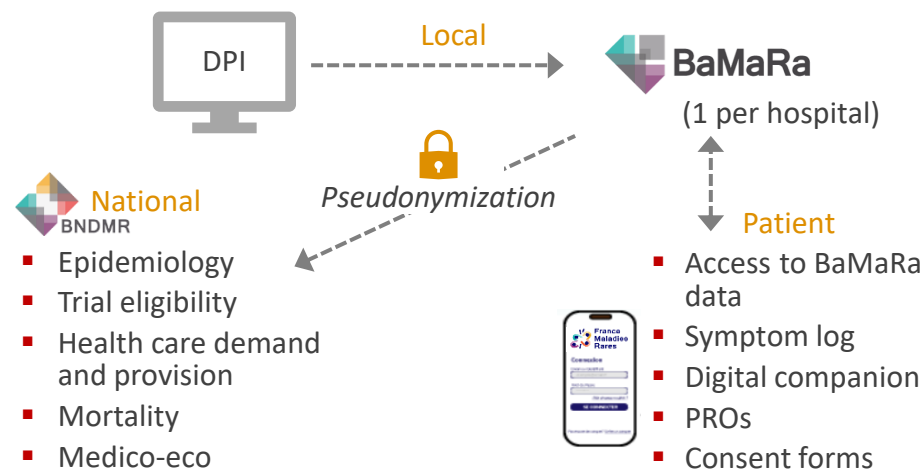
Funding

- Primary funding: **French State** (Ministry of Health)
- Integrated in the PNMR
- Additional **co-funding** from calls for projects (e.g., Convergence, ReuseRare)

Governance

- Steering authority: French Ministry of Health (DGOS)
- Operational lead: AP-HP (Health Data & Innovation Department)
- National network: expert centers and disease-specific networks
- Scientific and ethical oversight committees

Database description



Orphanet provides pharma companies with a valuable information resource to assess rare disease populations, identify expert centers, support clinical development and market access strategies



Rare diseases ecosystem – Orphanet

Presentation

- Free-access **database on rare diseases** and **orphan drugs**
- Initially created in France in 1997 by the INSERM
- Expanded since 2000s into a European and international consortium (~**40 countries**)

Objectives

- **Improve visibility** of rare diseases (standard nomenclature creation)
- Provide high-quality and validated **scientific information** (e.g., diseases, diagnostic tests, expert centers, treatments)
- **Support research, clinical care and policy-making**

Governance

- International network coordinated in France (INSERM, Paris)
- National teams collecting and validating local data (e.g., expert centers, labs, HCPs)
- Advisory boards (strategy, genetic data quality, national structures)

Database description

- Multi-service knowledge platform, including:

Rare diseases

- Disease encyclopedia
- Epidemiology and genetics

Other directories

- PAGs
- HCPs
- Institutions

Orphan drugs

- Marketed orphan drugs
- Drugs in development

Trials

- Ongoing research projects
- Registries and biobanks

Care resources

- Expert centers
- Hospitals and networks
- Diagnostic labs

Advanced tools

- ORPHAcode¹
- Orphadata²
- ORDO

Key data (2025)

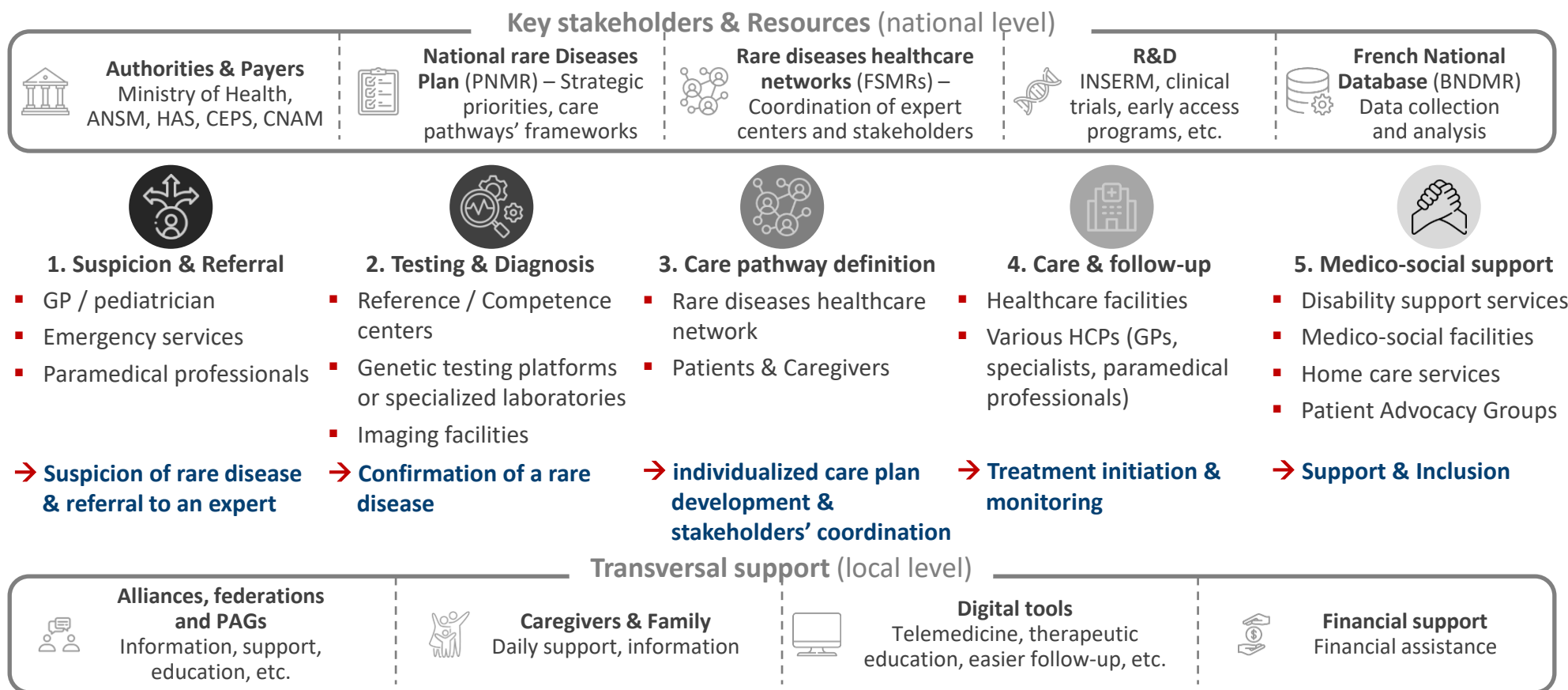
- **6,528 rare diseases described**
- 115,611 phenotypic annotations
- 16,418 **epidemiological data**
- **689 orphan drugs described**
- **8,648 expert centers**

Funding

- **Non-commercial** and publicly funded by:
 - INSERM
 - European Commission
 - National health institutions
 - Research and project contracts

Health authorities, HCPs and PAGs are involved in the patient's journey, from diagnosis to treatment and support, thus the need for a multi-level coordination of stakeholders

Patients – Rare diseases pathway



Sources: Filièresmaladiesrares.fr (as of Apr. 2026) – Rare diseases management, Ministry of Health, Families, Autonomy and Handicapped People (Mar. 2026) – Smart Pharma Consulting analyses

ANSM: National Agency for the Safety of Medicines and Health Products – BNDMR: National Rare Diseases Database – CEPS: Economic Committee on Healthcare Products – CNAM: National Health Insurance Fund – HAS: National Authority for Health – INSERM: National Institute of Health and Medical Research – PAGs: Patient Advocacy Groups – PNMRs: National Plans for Rare Diseases

Pharma companies must anticipate increasingly stringent and harmonized EU and local HTA requirements by strengthening early evidence strategies

Executive summary – Policy makers and payers (1/2)

	Situation	Key trends	Implications for pharma companies
Policy definition	- Structured rare diseases ecosystem built around successive national plans - Patient management highly organized - Strong institutional focus on: - Reducing diagnostic delay - Improving referral pathways - Integrating genomic medicine - Increasing role of European collaboration	Earlier and faster diagnosis driven by genomics, screening initiatives and better coordination between office-based and expert centers' physicians - Rapid expansion of data-driven healthcare - Growing importance of care pathway orchestration - Stronger EU-level integration	- Think beyond product: build a "system integration strategy" within the ecosystem - Map and engage early with expert centers, KOLs, networks members and PAGs Position the therapy as part of an improved care pathway Invest early in patient identification (e.g., biomarker testing, screening pathways) Integrate digital / data component into strategy
HTA	HTA led by the HAS, impacting pricing and reimbursement - For high-impact / innovative drugs, a CEESP assessment may be required - Strong emphasis on comparative clinical evidence, relevant comparators and clinical endpoints - Orphan drugs benefit from flexibility, but still face rigorous clinical scrutiny	EU HTA regulation: JCAs mandatory for orphan drugs from Jan. 2028 - Increasing expectations on robust comparative evidence - Rising importance of RWE to complement uncertainty at launch - Greater scrutiny on subgroup definition, durability of effect and relevance of endpoints	- Anticipate a dual layer: EU / French Seek early scientific advice to anticipate French HTA evidence requirements - If aiming for premium pricing, prepare early for economic and budget impact discussions - Plan for post-launch evidence generation (RWE) to reduce uncertainty

A drug's success will be based on its strong clinical value, early patient access and a sustained credibility through robust post-launch evidence generation, supported by continuous drug supply

Executive summary – Policy makers and payers (2/2)

	Situation	Key trends	Implications for pharma companies
Market Access	- Relatively favorable early access programs for innovative therapies - Complementary compassionate use pathways for urgent situations - Long-term access depends on HTA outcome, price negotiation and budget impact considerations - Real access also depends on patient identification, center readiness and prescribing behaviors	- Shift toward faster but more conditional access: - Available early access - Harder to sustain long-term value without additional data - Increasing demand for post-access RWE to confirm effectiveness, appropriate use and impact on the healthcare system - Stronger focus on budget impact and cost control	- Design a 2-step access strategy: Early access Sustainable reimbursement - Develop a France-specific RWE plan Define target population and eligibility criteria to ensure controlled uptake - Build a strong value narrative beyond price Anticipate discussions on budget impact, uptake speed and population size - Ensure operational readiness
Supply	Strict legal obligations on continuous supply: companies must ensure appropriate and continuous availability of drugs on the market - For medicines of major therapeutic interest, pharma companies must maintain safety stocks (two months / four in at-risk situations) - Obligation to notify authorities early in case of supply disruption	Expansion of national and EU-level supply security policies - Strong regulatory tightening after repeated shortages - Shift from soft enforcement to active sanctioning (financial penalties) - Increasing reputational risk, with supply performance becoming part of credibility with payers	- Implement shortage management plans and anticipate supply risks - Anticipate classification risk due to heavier obligations in case of MITM status - Need to integrate stock requirements into launch planning (not post-launch) - Accurate demand forecasting required, covering the risk of diagnosis expansion

Early engagement with rare disease networks, expert centers and data infrastructures is essential to improve patient identification, accelerate launch readiness and generate real-world evidence

Executive summary – Rare diseases ecosystem

	Situation	Key trends	Implications for pharma companies
Networks	23 national rare diseases networks to coordinate care, research and patient pathways - Include reference and competence centers, labs, researchers, PAGs	- Increasing role (care, R&D, data) - Stronger integration with European Reference Networks (ERNs) - Growing role in data collection and pathway standardization	- Engage at network level - Key entry point for patient identification & referral flows - Opportunity to co-develop tools - Critical for launch execution
Expert centers	- Highly structured hospital-based system with reference (expertise hubs) and competence centers (local care delivery) >2,000 centers across France	- Increasing expertise centralization - Stronger coordination between national & local levels - Expansion of telemedicine - Key role in clinical trials & cohorts	- Primary prescribers and influencers - Essential for clinical development & post-launch evidence - Need to support diagnosis acceleration initiatives - Critical for patient onboarding and adherence
Registries	- National database: BNDMR collects standardized patient data - Numerous disease-specific registries - Data used for epidemiology, research and policy-making	- Shift towards RWE and PROs as decision drivers - Increasing integration with HTA and reimbursement decisions - Digitalization & interoperability	- Post-launch data collection required - Need early investment in registries & data partnerships - Critical for pricing, reassessment and lifecycle management
R&D centers	- Strong public-private ecosystem: academic hospitals, institutes, etc. - Close link between clinical care and research (same centers)	- Growth of public-private partnerships - Increased use of genomics & precision medicine	- Collaborate with academic centers - Opportunity for co-development & repositioning strategies - Integrate R&D + access + data strategy

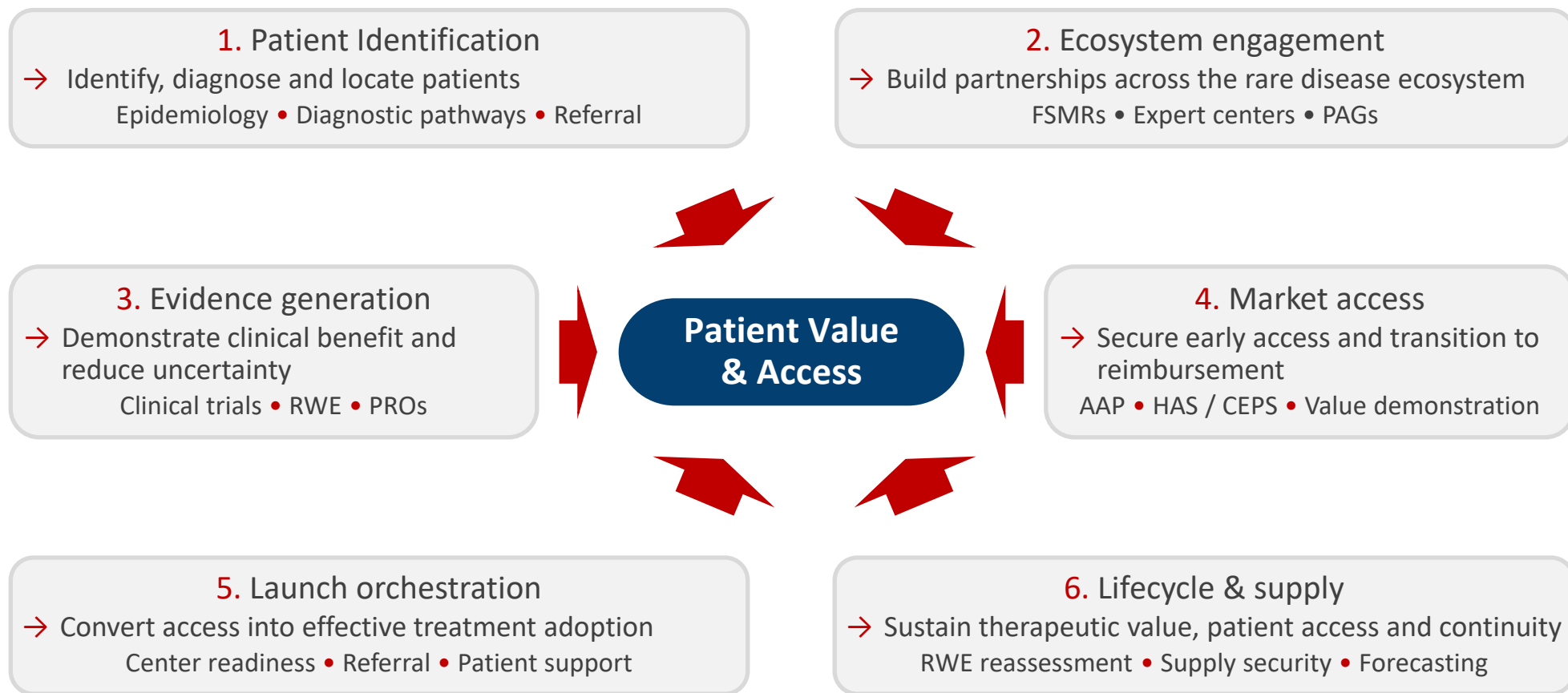
Engaging patients, caregivers and PAGs helps pharma companies better understand unmet needs, address diagnostic barriers and generate meaningful patient-centered evidence

Executive summary – Patients & PAGs

	Situation	Key trends	Implications for pharma companies
Patients	~3 million people affected by rare diseases (fragmented, ultra-small populations) Long diagnostic journey Follow-up via expert centers and networks - Increasing digital inclusion (e.g., registries) - Demand for faster and equitable access	- Becoming active participants in care (shared decision-making) - Increasing contribution to real-world data (PROs, registries) - Higher expectations on quality of life and treatment convenience	- Demonstrate meaningful patient benefit beyond clinical endpoints (Quality of Life, daily functioning) - Integrate patient-reported outcomes early in development - Ensure ease of use and adherence support (critical for oral drugs)
Caregivers & Family	Central role in rare diseases (especially pediatric and severe conditions) - Often responsible for treatment management, coordination of care and daily support - High physical and emotional burden - Increasing recognition in health policies	Growing visibility of caregiver burden in policy and research Inclusion of caregiver perspective in quality-of-life assessments - Demand for better support tools and simplification of care pathways	- Need to demonstrate impact on caregiver burden (time, stress, complexity) Opportunity to develop support services Simplify treatment pathways
PAGs	Highly structured and influential, connected to expert centers and national networks - Central role in patient identification, awareness and reducing diagnostic delay, strong advocates for rapid and equitable access to innovation Strong credibility with clinicians / policy makers	- Growing involvement in research, healthcare policy and patient-centered initiative - Increasing role in data generation - Stronger European coordination - Focus on real access and equity	Engage PAGs early, treat them as strategic partners Co-develop initiatives around disease awareness, diagnosis and patient journey mapping - Ensure transparent, compliant and long-term relationships

Winning in the French rare diseases market requires orchestrating six complementary capabilities around patient value and access

Six capabilities to win



Consulting firm dedicated to the pharmaceutical sector operating
in the complementary domains of strategy, management and organization

Market Insights Series

- This series provides practical tools and recommendations to enhance effectiveness and efficiency of the most important activities or processes in place within pharma companies
- Our tools and recommendations are based on both:
 - Our consulting experience in the pharma sector
 - Our research for innovative, pragmatic and useful solutions
- Each issue is designed to be read in less than 20 minutes

French **Rare Diseases Market** 2026 & Beyond Ecosystem Challenges & Implications for Pharma Companies

- Developing a strategy for rare diseases in France requires a clear understanding of the care ecosystem, key opinion leaders and regulatory requirements
- This paper is a synthetic review of the situation analysis, the key trends and the implications for pharma companies
- Smart Pharma Consulting can help pharma companies size a market and identify priority stakeholders to develop strategies tailored to their assets and objectives...
- ... supported by tactics for a practical launch plan

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1. Market Insights	5. Marketing
2. Strategy	6. Sales Force Effectiveness
3. Market Access	7. Management & Trainings
4. Medical Affairs	
- Our research activities in pharma business management and our consulting activities have shown to be highly synergistic
- We remain at your disposal to carry out consulting projects or training seminars to help you improve your operations

Best regards

Jean-Michel Peny