# Advanced Therapy Medicinal Products - ATMPs in Brazil: an overview and pricing strategies

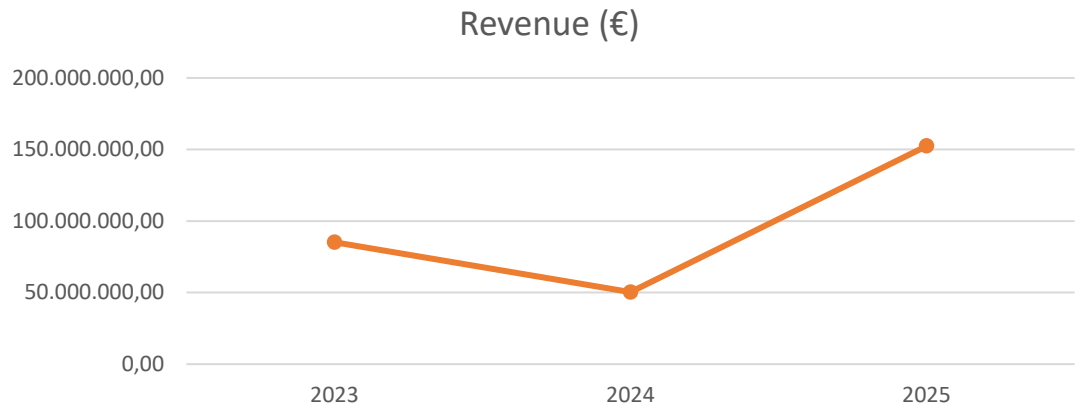
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# ATMPs companies revenue

| Brand | API | Laboratory | Ex-factoy price(2026)* |
|---|---|---|---|
| Carvykti | citacabtageno autoleucel | Janssen - Cilag Farmacêutica | € 406.683,26 |
| Kymriah | tisagenlecleucel | Novartis Biociências S.A | € 279.489,47 |
| Luxturna | voretigeno neparvoveque | Novartis Biociências S.A | € 307.759,55 |
| Zolgensma | onasemnogeno abeparvoveque | Novartis Biociências S.A | € 1.443.667,25 |
| Yescarta | axicabtagene ciloleucel | Laboratório Kite | € 314.941,51 |
| Tecartus | brexucabtageno autoleucel | Gilead Sciences Farmaceutica do Brasil Ltda. | € 279.489,34 |
| Elevidys** | delandistrogeno moxeparvoveque | Produtos Roche Químicos e Farmacêuticos S.A. | € 2.521.838,75 |

Source: Federal Government
*Currency Exchange: €1,00 ≈ R$6,00
**Registration cancelled by Anvisa.




| Category | Revenue 2025 (EUR) | 2024x2025 (Growth) |
|---|---|---|
| All | € 30.466.702.722,05 | 13.73% |
| Biological | € 9.503.056.987,70 | 17.52% |
| Specific (some vitamins, minerals and others classified as medicine | € 1.825.732.464,51 | 6.95% |
| Phytotherapeutic | € 157.290.236,45 | 14.40% |
| Generic | € 4.196.701.484,13 | 16.74% |
| New | € 10.109.230.315,68 | 14.16% |
| Branded generics | € 4.522.271.949,83 | 3.78% |
| ATMPs and Radiopharmaceuticals | € 152.419.283,74 | 202.75% |

Source: https://www.gov.br/anvisa/pt-br/assuntos/noticias-anvisa/2026/cmed-divulga-9deg-edicao-do-anuario-estatistico-do-mercado-farmaceutico

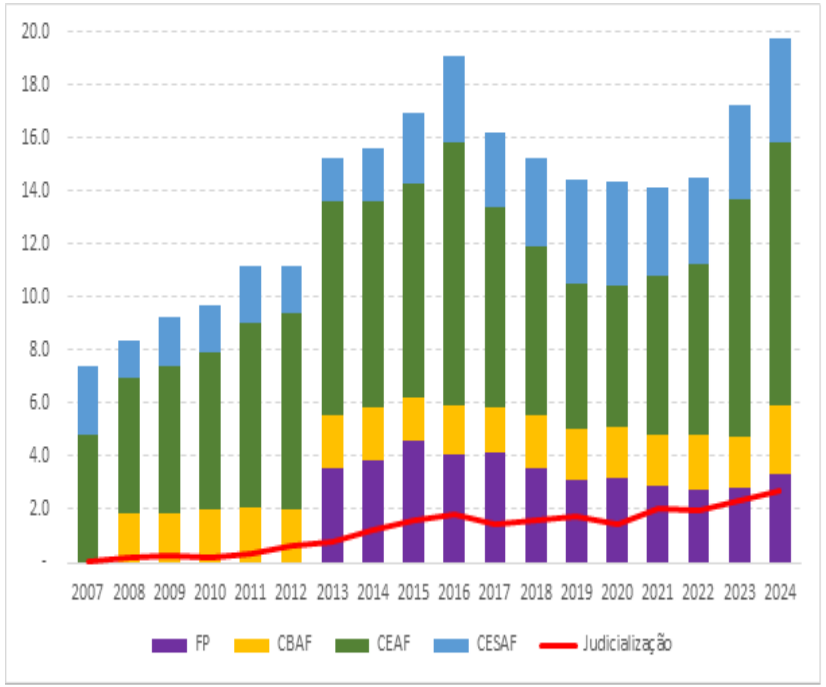
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# Judicialization impact

- The judicialization of healthcare puts pressure on the public budget and threatens the sustainability of the systems.
- € 450 million* in 2024 was spent on fewer than 6,000 patients.
  - ❖ Elevidys case: € 250 million* to comply with 55 lawsuits:
    - ➢ 38 had the same prescribing physician;
    - ➢ 35% of the lawsuits involved patients outside the age range indicated in the drug's leaflet.
- Judicialization can become a market strategy, creating a "parallel path" for commercialization.

*Currency Exchange: €1,00 ≈ R$6,00

**Chart 1 - Health Budget for medicines and federal government spending on litigation - Values in billions of reais (R$), updated to 2024**




Source: https://www.gov.br/anvisa/pt-br/assuntos/medicamentos/cmed/Regulacao/arquivos/RelatriodeAIRDemandasJudiciais.pdf/@@display-file/file

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# Projected budgetary impact of ATMPs in the Unified Health System (2024 – 2029)

## Projection of total population, live births, eligible patients and Official Inflation rate

| Variable | Scenario | 2024 | 2025 | 2026 | 2027 | 2028 | 2029 |
|---|---|---|---|---|---|---|---|
| Population | | 205.198.287 | 206.265.318 | 207.337.898 | 208.416.055 | 209.499.818 | 210.589.218 |
| Live Births | | 2.573.634 | 2.587.017 | 2.600.469 | 2.613.991 | 2.627.584 | 2.641.248 |
| Official inflation rate (IPCA) | | 3,91% | 3,50% | 3,50% | 3,50% | 3,50% | 3,50% |
| Eligible Patients | **Conservative** | 1.595 | 1.603 | 1.611 | 1.620 | 1.628 | 1.637 |
| | **Moderate** | 1.782 | 1.791 | 1.800 | 1.810 | 1.819 | 1.829 |
| | **Aggressive** | 2.143 | 2.154 | 2.166 | 2.177 | 2.188 | 2.200 |

| Scenario | Case i - immediate incorporation of all ATMPs* | Case ii - Incorporation of all ATMPs one year after analyses * | Case iii - gradual incorporation of ATMPs one year after analyses* |
|---|---|---|---|
| Conservative | € 4,72 | € 4,68 | € 2,78 |
| Moderate | € 5,92 | € 5,85 | € 3,48 |
| Aggressive | € 8,87 | € 8,78 | € 5,23 |

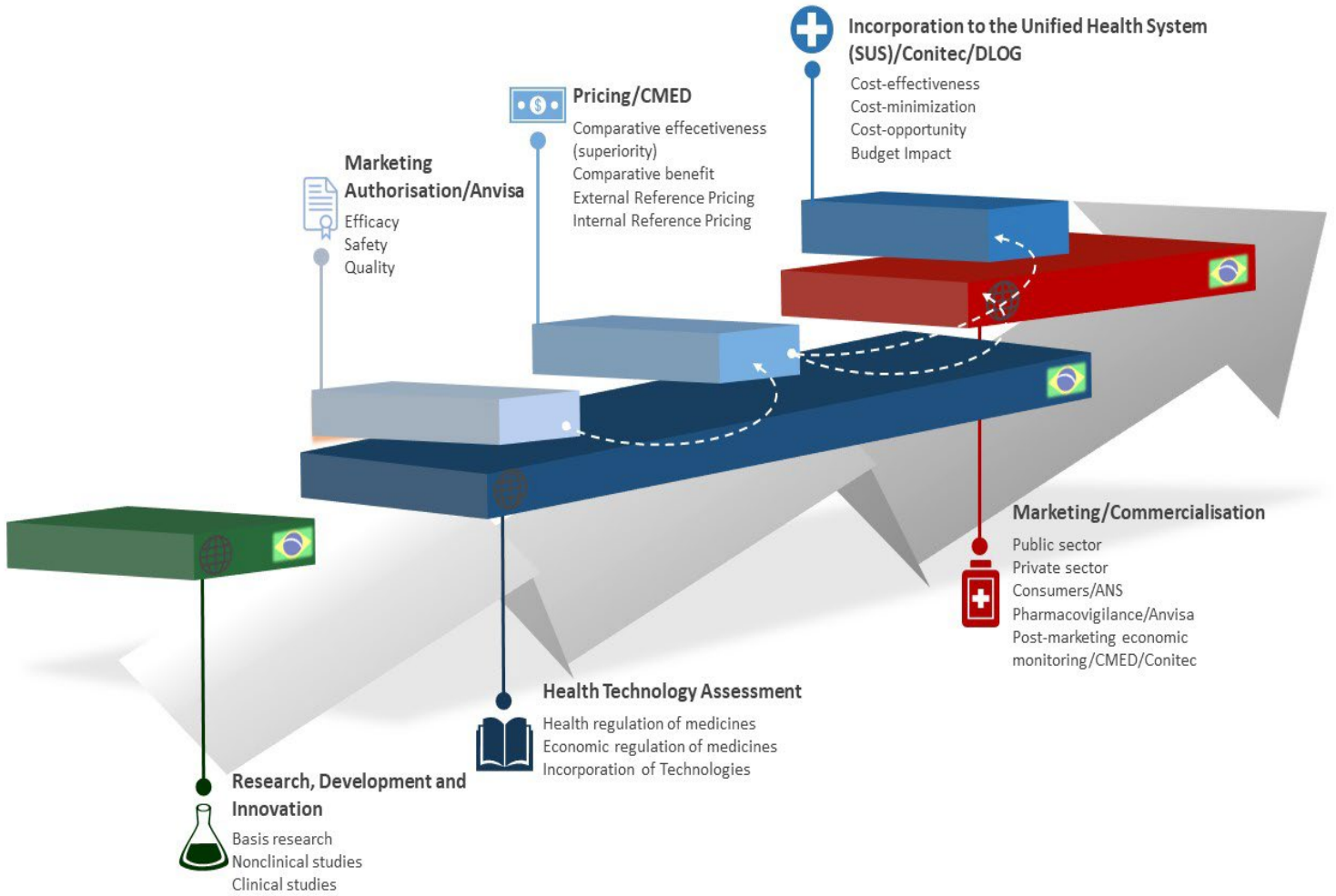
*Billion Euros

Currency Exchange: €1,00 ≈ R$6,00

Source: Araujo DV, Sachetti CG, Carvalho ACC, Oliveira G, Saqueto L, Pelizzaro A, Ma cedo A, Mussi JP, Heck MP, Maia JP. Impacto Orçamentário dos Produtos de Terapias Avançadas no Sistema Único de Saúde. JAFF [Internet]; 2024; 9(3): doi: 10.22563/2525-7323.2024.v9. n.3. p.72-84

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# Pharmaceutical assessment flow chart in Brazil



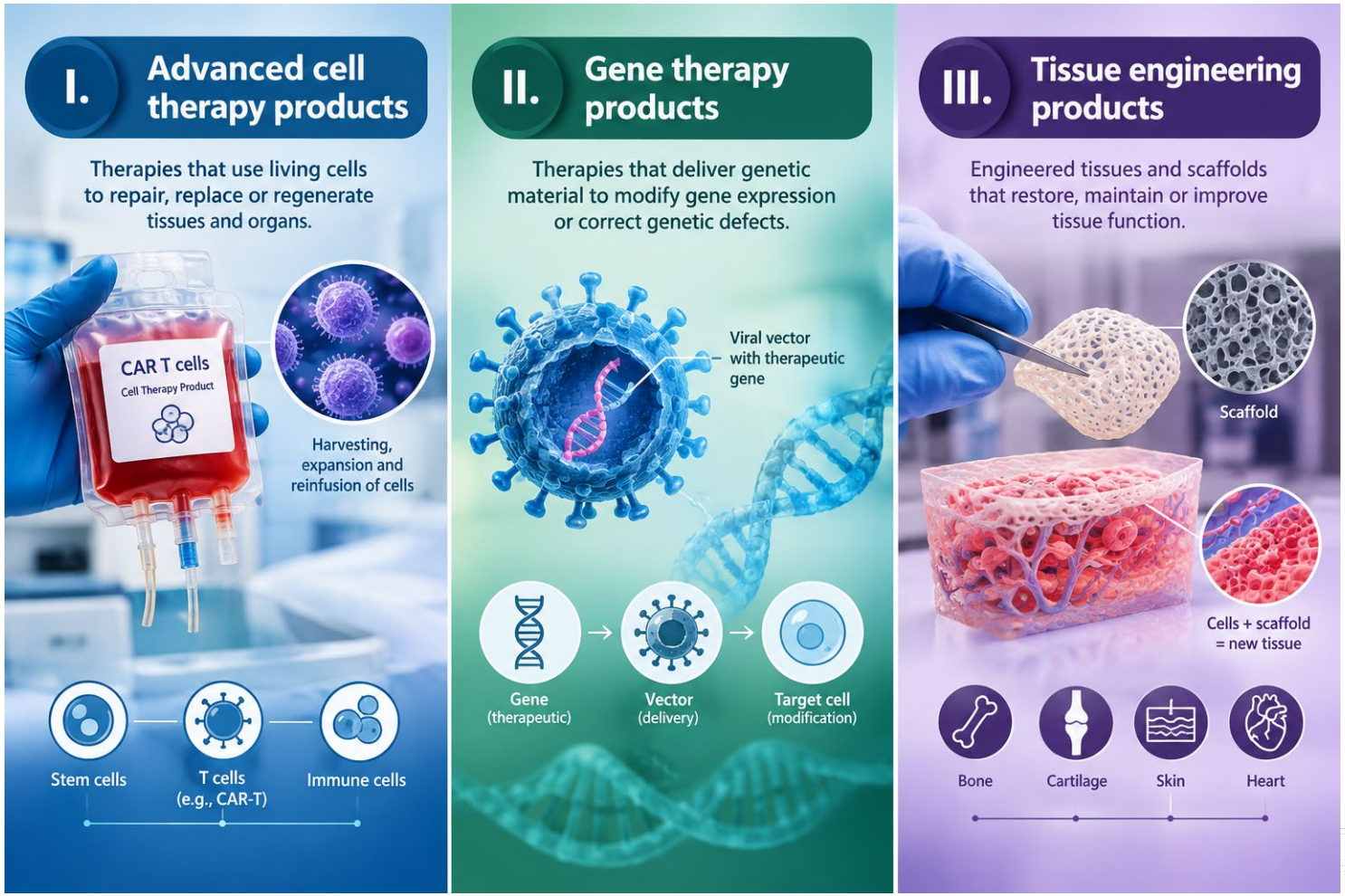

ANS: National Agency of Supplementary Health; Anvisa: National Health Regulatory Agency; CMED: Drug Market Regulation Chamber; Conitec: National Committee for Health Technology Incorporation to the Unified Health System; DLOG: Logistics Department/Ministry of Health; SCMED: Executive secretariat of CMED; SUS: Unified Health System.

Modified from: Brummell AMI, Ronchini MAK, Pingret-Kipman D, Rego FM, Santana JR. **Pharmaceutical pricing and reimbursement policies.** *4th PPRI Conference Medicines Access Challenge*. Vienna, Austria. 2019.

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# Types of Advanced Therapy Products




Source: Resolução da Diretoria Colegiada - RDC nº 505, de 27/05/2021

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# ATMPs pricing currently

- Our new regulation (RESOLUÇÃO CM/CMED Nº 3, DE 29 DE DEZEMBRO DE 2025) does not comprise specific requirements or calculation method for ATMPs pricing.
- Currently, ATMPs are framed as **Omitted cases*** by the new regulation until a specific regulation for ATMPs pricing is published.
  - ➢ That brings uncertainty and less predictability to companies;
  - ➢ Discourage Investments;
  - ➢ Late launching;
  - ➢ On the other hand, we have more flexibility to define the best technique for pricing calculation, such as:
    - ✓ Value-based techniques;
    - ✓ ERP;
    - ✓ Cost of treatment if a national comparator is available.




*Omitted cases: Cases not covered by the standard pricing categories

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# New ATMPs pricing regulation (in progress)

1. CMED has published a call for contributions on ATMPs pricing (Edital de Chamamento 01/2025): from feb/10th to mar/28th.
   - Objective: to collect data, information, opinions, and suggestions regarding pricing criteria for ATMPs;
   - Targeted stakeholders: healthcare professionals, patients, pharmaceutical industry representatives, researchers, government entities and all other interested parties;
   - Pricing advanced therapies involves specific challenges and directly impacts the viability of these treatments. Thus, contributions from society will help build an appropriate regulatory instrument, promoting a more transparent, participatory and innovation-friendly regulatory environment.
2. We've promoted a workshop, in March 2026, to present and discuss value-based pricing approaches for ATMPs, such as the Pharmaceutical Innovativeness Index (PII) and the efficiency frontier;
3. A working group was created to discuss alternatives, to draft a proposal and to promote regulatory impact analyses. A public consultation is planned for late this year.




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# HTA Criteria Under Discussion

- More important than ever!!
- Criteria being discussed:

I – Added Therapeutic Value (ATV);
II - Unmet therapeutic need (UTN);
III - Existence of therapeutic alternatives;
IV - Methodological quality and risk of bias in the evidence;
V - Design of scientific studies;
VI - Efficiency gains for the health system, through the reduction of costs associated with treatment administration;
VII - Accessibility;
VIII - Epidemiological data;
IX - Innovative activity; and
X - Other therapeutic, social, economic, structural, organizational, individual, or ethical factors, provided they are adequately justified, relevant, and consistent with the analysis of the case in question.




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# Pricing strategies being discussed

- Approaches under discussion:
- External Reference Pricing (ERP);
- Value-based methodologies:
  - Pharmaceutical Innovativeness Index (PII) and the efficiency frontier;
- Price revisions;
- pharmacoeconomic analysis:
  - cost-effectiveness;
  - cost-minimization;
  - cost-utility; and
  - cost-benefit studies
- Cost of treatment;
- Managed entry agreements (MEA) and provisional prices;
- Risk-sharing agreement (RSA)

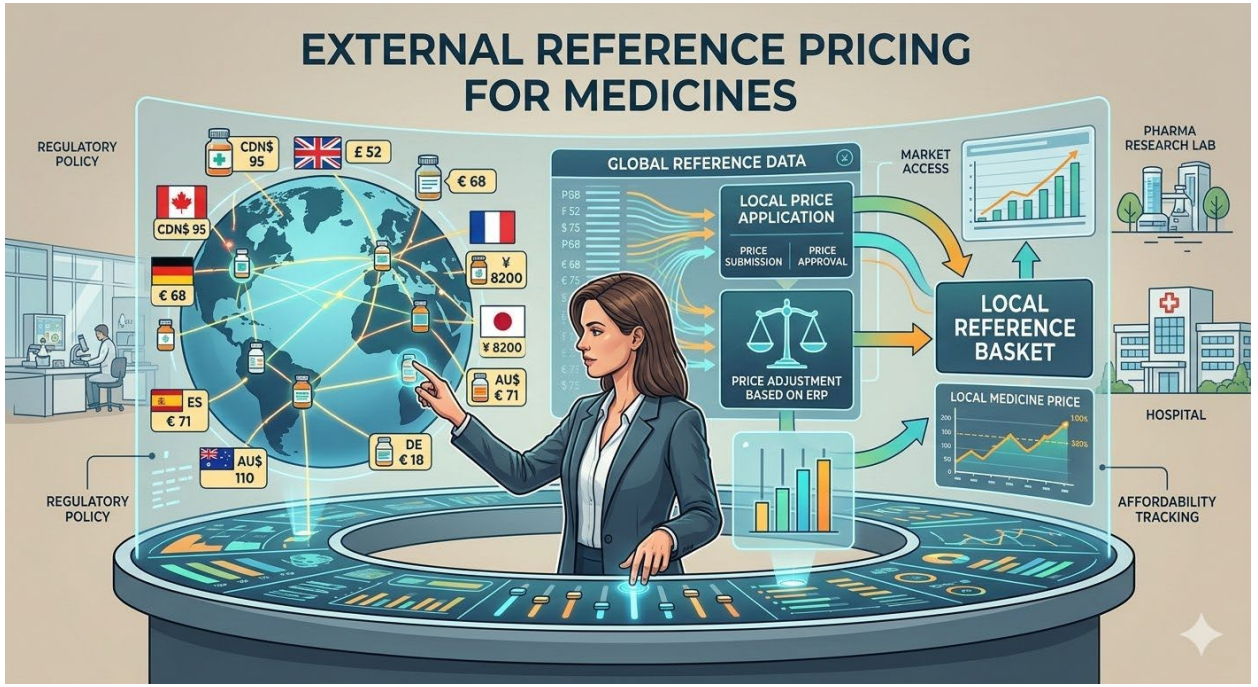



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# External Reference Pricing - ERP aspects

- Our Basket: South Africa; Germany; Australia; Canada, Spain, United States, France, Greece, Italy, Japan, Mexico, Norway, Portugal, United Kingdom and the Origin.
- It must have price at least in 4 countries, otherwise a provisional price is established and revised every year at least.
- The lowest price at the ex-factory price level.
  - ❖ External reference pricing relies on published list prices.
  - ❖ List prices may differ substantially from net prices after discounts and negotiations.
  - ❖ These differences limit how well reference prices reflect actual expenditure.
  - ❖ Pricing decisions should account for this limitation.




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# Managed Entry Agreements – MEA

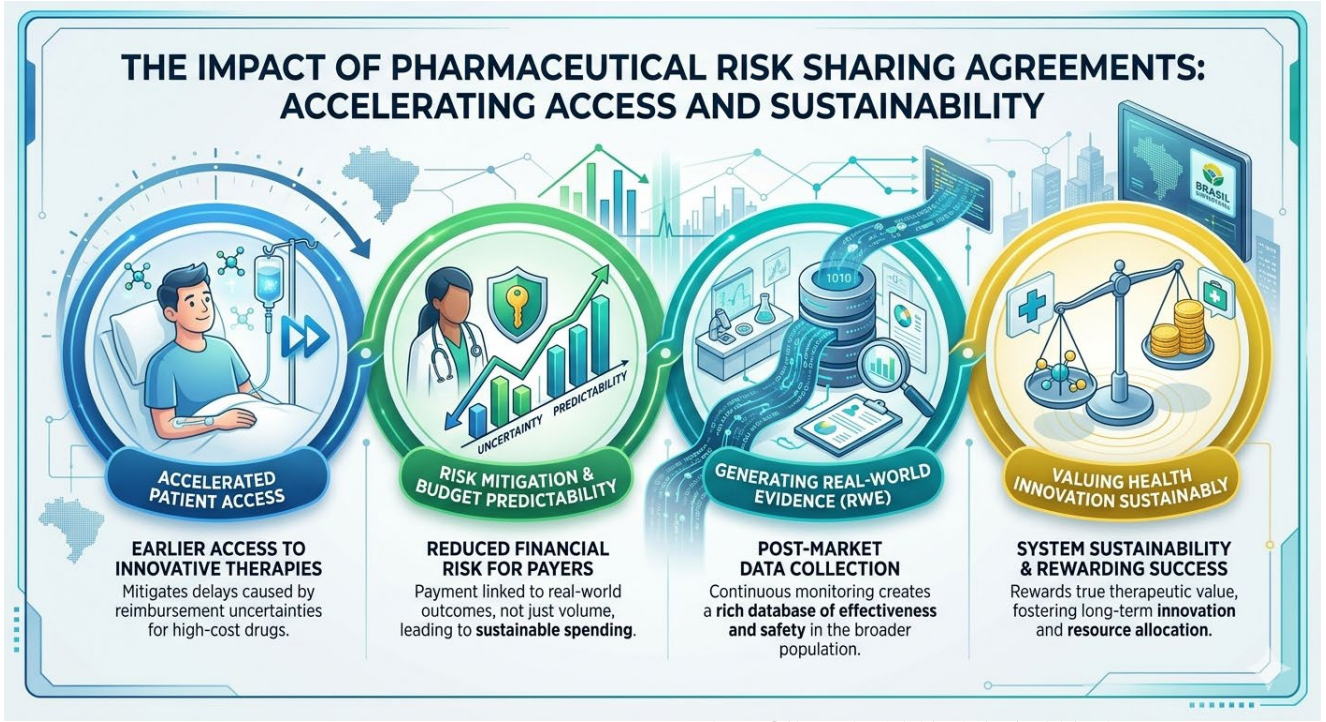
- Anvisa: Conditional marketing authorization requires the submission of additional evidence.
- CMED: A provisional price may be established when information needed for definitive pricing is not yet available.
- Conitec and Ministry of Health: Managed entry agreements may support earlier access within our Unified Health System while addressing clinical or financial uncertainties.




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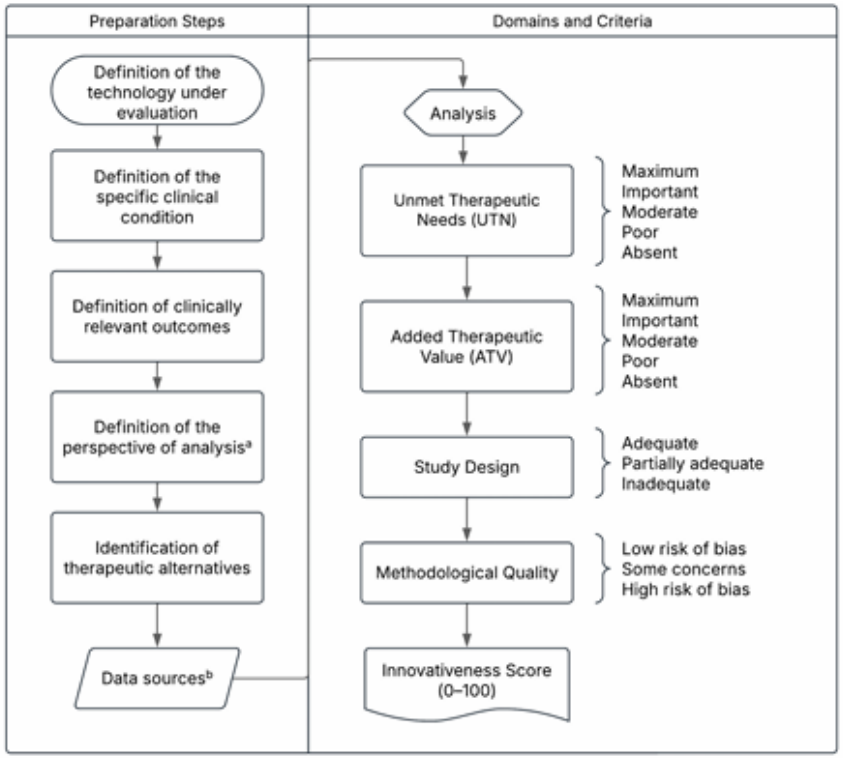
# Risk Sharing Agreements - RSA

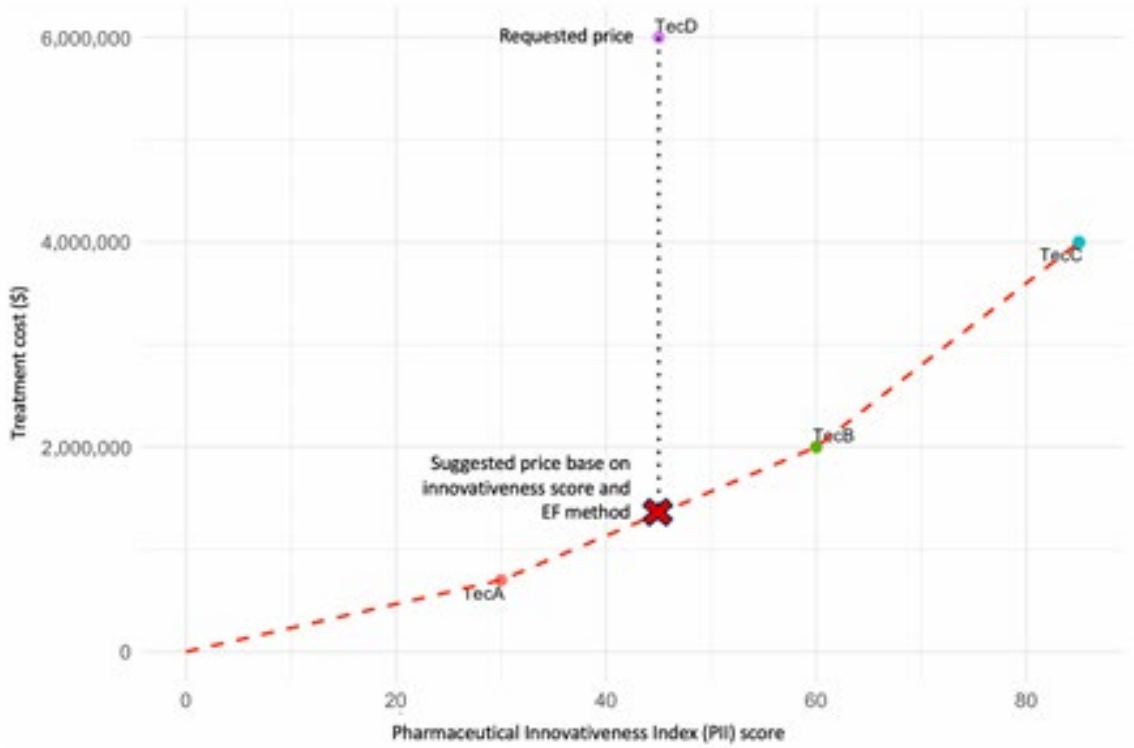
- Risk Sharing Agreements – RSA are being developed by Conitec and Health Ministry. That was first tested for Spinraza (nusinersen) for the treatment of Spinal Muscular Atrophy (SMA 5q) and, more recently for Zolgensma (onasemnogeno abeparvoveque).
- Some terms of the agreement:
  - ❖ Partial payment spllitted in 5 Years (20% per year);
  - ❖ Refunds, rebates and payment suspension if patients fail to respond or disease progresses (Performance-Based);
  - ❖ Companies must cover additional costs, if the number of patients surpasses the number previously informed.
  - ➢ Advantages:
    - ❖ Early Access: Patients get innovative, high-cost therapies faster before long-term data is fully established.
    - ❖ Budget Control: Payers reduce the financial risk of paying for ineffective treatments.
    - ❖ Real-World Evidence: Encourages tracking actual drug performance outside controlled clinical trials.




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# Value-Based Pricing: The Pharmaceutical Innovativeness Index (PII) and Efficiency Frontier




Source: Gargano,L.P.; Borin, M.C.; Prata, W.M.; Alvares-Teodoro, J.; Acurcio, F.d.A.; Iunes, R.F.; Guerra, A.A., Jr. The Pharmaceutical Innovativeness Index: Supporting Value-Based EconomicRegulationof Innovative Medicines. J. Mark. Access Health Policy 2025, 13, 60. https:// doi.org/10.3390/jmahp13040060

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# Thank you!

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