Machado Meyer

BRAZILIAN REGULATORY FRAMEWORK FOR RARE DISEASES

WHICH ARE THE MAIN BRAZILIAN HEALTH AUTHORITIES?

Specifically with regard to rare disease drugs, including gene and cell products, the following entities play a relevant role on a pre-market level:

Brazilian National Commission for Research Ethics (CONEP)	Jointly with the Brazilian National Health Council (CNS), provides for regulations applicable to human clinical research, as well as evaluating and participating on public health policies. CONEP may also authorize certain clinical study protocols depending on the features involved.
Local Ethics Committees (CEPs)	Organized within health institutions and are responsible for approving clinical protocols on a local level.
National Health Regulatory Agency (ANVISA)	Set forth sanitary rules, procedures and standards regarding safety, quality and efficacy of drugs and medical devices (including software). May also regulate other matters such as labelling, recall actions and health-related services.
Market Chamber Regulation for Drugs (CMED)	Provides for drug pricing rules.

Brazilian Ministry of Health (MoH)	Designs, coordinates and executes public health policies – including the operationalization of the public health system (SUS) – which serves around 75% of the Brazilian population. It also provides for a National Policy for People with Rare Diseases.
Brazilian National Commission for the Incorporation of Technologies into the Public Health System (CONITEC)	Technical entity linked to the MoH and responsible for assessing the availability of evidence-based medicine versus cost-effectiveness and budget impacts of a certain technology or product within the SUS.

LEGAL DEFINITION

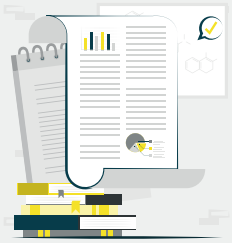
A **rare disease** is defined as a condition that affects up to sixty-five (65) people in one hundred thousand (100.000) individuals, based on official national data or, when non-existent, on data published in technical-scientific documentation.

FROM RESEARCH TO THE PATIENT – MAIN STEPS

As a general rule, a drug clinical trial and marketing application must comprise information regarding:

- A description of the rare disease for which the drug is indicated.
- Drug's relevance for treatment, diagnosis or prevention purposes.
- Global and national data on the prevalence and incidence of the rare disease for which the drug will be indicated.
- Documentation proving the drug's designation for a rare disease by a foreign regulatory authority, if available.

Only a duly licensed Brazilian entity may hold a drug marketing authorization with Anvisa.



Drugs indicated for rare diseases or seriously debilitating conditions may be subject to a priority review in situations where there is no therapeutic alternative available, or when there is a significant improvement in safety, efficacy or adherence to treatment. Deadline of analysis is ~120 days.

WHAT DOES IT MEAN TO HAVE A DRUG INCORPORATED INTO THE SUS?

Drugs and other technologies deployed into the SUS are financed by taxes and other forms of renevue to the government on a federal, state and local levels, according to the responsabilities of each entity.

The incorporation, exclusion and/or change of drugs in the SUS list are, as a rule, attributions of the MoH, assisted by CONITEC. Moreover, both are responsible for establishing clinical protocols and therapeutic guidelines for dispensing SUS drugs, according to:

(i) the different stages of the relevant disease or condition;

(ii) safety, efficacy and quality factors of available drugs; and

(iii) alternatives in case of intolerance or relevant adverse reaction of each drug, product or procedure considered to be of first choice.

HOW DOES THE BRAZILIAN GOVERNMENT PURCHASE DRUGS?

In the healthcare space, the MoH is the main authority responsible for engaging on public procurements involving health-related products to be deployed in the SUS, provided that such technologies are:

- Duly regularized with Anvisa to be locally manufactured, imported, distributed and/or commercialized; and
- Incorporated into the SUS by CONITEC.

As a rule, public purchases must be contracted by public bidding proceedings so to ensure equal conditions to all bidders, promote competition among interested entities, and elect the most qualified player to serve the public interest. However, the Public Procurement Law provides for circumstances in which the Brazilian government may directly procure with a private company, either by means of a waiver on public bid proceeding ("dispensa de licitação") or non-requirement of bid proceeding due to unfeasibility of competition ("inexigibilidade de licitação").

The main difference is that, in the first, there is a possibility of competition that justifies the bid, but Public Procurement Law expressly provides for exceptions to such general rule that allows a direct procurement. On the other hand, the non-requirement scenario happens when there is only one product or one bidder that meets the necessities of the Brazilian government, making the bidding fruitless. Notwithstanding the above, in both cases the Brazilian government must still justify the agreement's price and the underlying reasons regarding the choice of a certain contractor under a previous administrative proceeding to be conducted by the public entity in charge.

WHAT ARE THE NECESSARY STEPS FOR A FOREIGN BIOTECH COMPANY TO GET THEIR DRUG INTO THE SUS?

I	Biotech's submission of a clinical trial authorization (when performed in Brazil).	
II	CEP/CONEP ethical approval of the clinical research (when performed in Brazil).	**30 ~ 60 days**
III	Clinical trial authorization by Anvisa (if applicable).	**~ 90 days**
IV	Drug marketing authorization approval by Anvisa.	**~ 120 days**
V	Drug price definition by CMED.	**~ 90 days**
VI	Technical assessment regarding incorporation of the drug into the SUS by Conitec.	**~ 180 days**
VII	Drug offer to SUS patients.	**~ 180 days**

total of 690 ~ 720 days.

! Other timelines may apply for incorporation of drugs and treatments into the Brazilian private healthcare system.

GET IN TOUCH

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