Pharmaceutical Regulations of Advanced Therapy

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Advanced Therapy

Agência Nacional de Vigilância Sanitária - Anvisa

- Anvisa’s role as an integrant part of the Unified Health System.

- Themes where we have regimental competence and regulatory tools for action: exceptional imports, access to drugs at research stage, approval of clinical protocols.

- Principles: EFFICIENCY AND TRANSPARENCY.

New Regulatory Benchmarks

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- **Prioritization of the technical analysis of requests – RDC No. 37/2014, published on the Federal Official Gazette of 16 June 2014,** provides for the prioritization of the technical analysis of requests for registration, post-registration, and previous authorization for clinical trials with drugs and biological products:

  - **Registration requests:** drugs used for rare, neglected, emerging, or re-emerging diseases, among others.

  - **Clinical research:** with drugs used for rare, neglected, emerging, or re-emerging diseases; conducted exclusively on **pediatric and adolescent population**, among others.

  - **Response period:** 75 days for registration requests and 45 days for previous authorization.

http://bvsms.saude.gov.br/bvs/saudelegis/anvisa/2014/rdc0037_16_06_2014.pdf

New Regulatory Benchmarks

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- **Registration of New Drugs Biological – RDC No. 55/2010** :

➢ *Art. 23. Exceptionally, the company may require new biological product registration used in the treatment or prevention of serious diseases and / or high mortality, with Phase II clinical studies already completed and phase III studies in progress, provided it is demonstrated a high therapeutic or preventive efficacy and / or there is no other comparable alternative drug or therapy for that disease stage.*

§ 1. If the registration granted by ANVISA, the safety and efficacy should be monitored and continuously evaluated in Brazil, the company's pharmacovigilance system which holds in view of the current legislation.

§ 2 In the cases referred to in this article in addition to the documentation described in Sections I and II of Chapter III of this Resolution, in the registration application protocol act, the applicant company must submit the following documents:

I - performing schedule and completion of clinical trials phase III;

II - Preliminary results of clinical phase III studies, if available.

§ 3. The results of the clinical phase III studies must be submitted to ANVISA as soon be available, as highlighted by the realization schedule.

http://bvsms.saude.gov.br/bvs/saudelegis/anvisa/2010/rdc0055_10_10_2010.pdf

New Regulatory Benchmarks

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- **Registration of New Drugs – RDC No. 60/2014** :

  - *Article 25. The company may present,* ***as an exception, the report of clinical trials including concluded phase II studies*** *and initiated phase III studies aiming at requesting the registration of a new drug intended for preventing or treating* ***severely life-threatening or highly debilitating diseases, as long as an unmet medical need is confirmed for both cases.***

    Sole paragraph. ***In specific cases,*** *where phase III studies are not applied and phase II studies are enough to confirm the drug's efficacy and safety,* ***the company may submit the registration request after phase II studies are concluded.***

http://bvsms.saude.gov.br/bvs/saudelegis/anvisa/2014/rdc0060_10_10_2014.pdf

New Regulatory Benchmarks

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- **Approval for Clinical research on drugs – RDC No. 09/2015:**

  - Defines procedures and requirements for the conduction of clinical researches on drugs, and implements a new assessment model based on the clinical development plan – Drug Clinical Development Dossier (DDCM, in Portuguese).

  - Defines deadlines for the approval of protocols:

    - *Article 36. After receiving the DDCM, Anvisa will assess it in up to 90 (ninety) calendar days. (...)*

    - *Paragraph 3. Exceptions to the provisions in the caption and first paragraph are the submissions of clinical development in at least one of the following situations: national development, clinical development of biological products – including vaccines – and clinical development in phase I or phase II. For such cases, the technical area will assess the DDCM in up to 180 (one hundred and eighty) calendar days after Anvisa receives the DDCM, and the clinical trial may only begin after Anvisa's approval.*

  - Simplifies the import procedures of products under investigation.

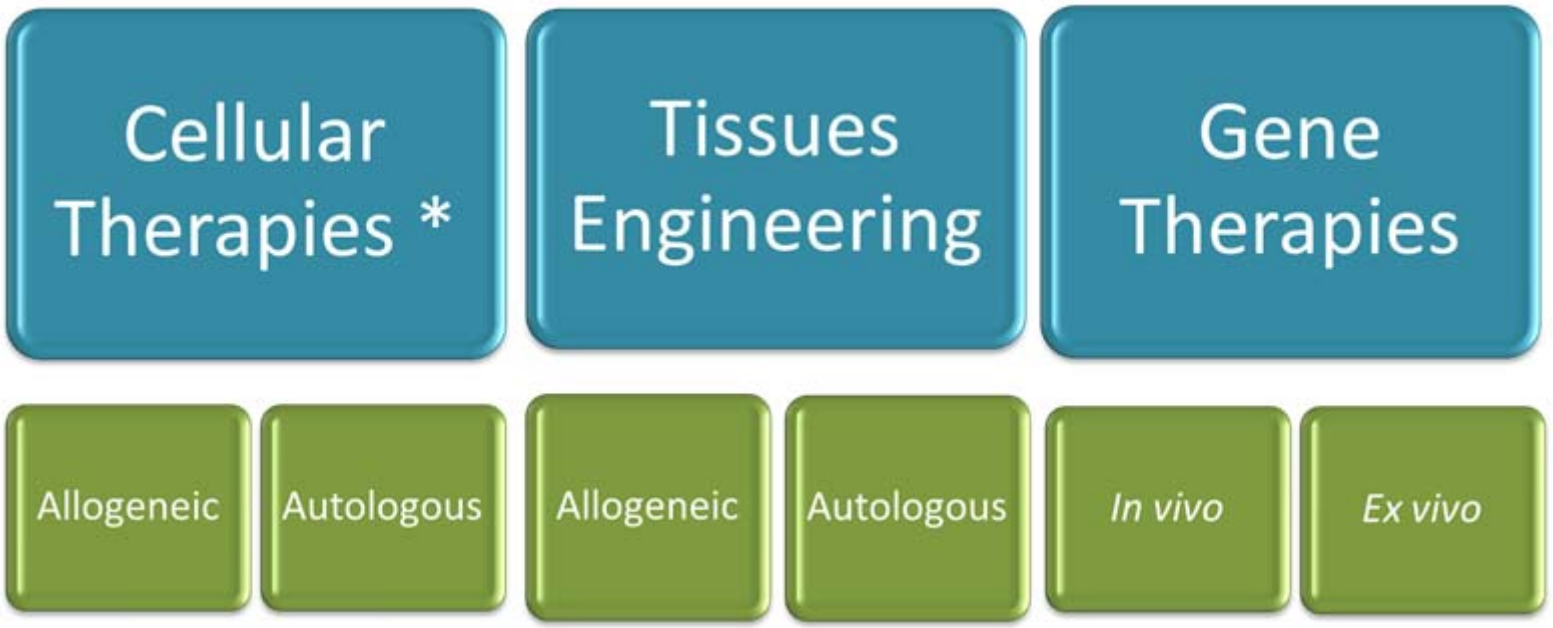
http://www.anvisa.gov.br/areas/coges/legislacao/2015/RDC_09_2015.pdf

New Regulatory Benchmarks - perspectives

Subject included in Anvisa's Regulatory Agenda 2015/2016:

Theme 59 – Clinical Trials of Advanced Therapies

Theme 60 – Market Authorization of Advanced Therapies



* except minimally manipulated Cells for homologous use
e.g: bone marrow transplant

Other initiatives

Improvement of the activities of approval of clinical protocols and registration

•SUMED restructuring: Efficacy and safety/ Pharmaceutical Technology – enabling technical improvement per area of knowledge – analysis stratification.

•Pre-submission meetings with the companies and further dialogue for technical discussions – reducing the need of technical requirements and the period for analysis – (Administrative Rule No. 219/SUMED/ANVISA dated 23/Feb/2015).

•Establishment of partnerships with medical societies, as well as with scientific development entities, such as CNPq and others.

•Promotion of technical discussions for the more complex drug cases with the presence of experts, at the Drug Technical Chamber (CATEME) and the Scientific Health Surveillance Commission (CCVISA).

Other initiatives

Transparency/ International Regulatory Convergence

- Publication of the technical and scientific bases for registration grant and denial. (since March 2015)
  http://www.anvisa.gov.br/datavisa/Fila_de_analise/index.asp

- Publication of the approved clinical trials on Anvisa's website.
  http://portal.anvisa.gov.br/wps/content/Anvisa+Portal/Anvisa/Inicio/Medicamentos/Assunto+de+Interesse/Pesquisa+clinica/Consulta+de+ensaios+clinicos+autorizados+pela+Anvisa

- **Participation on the ICH working groups.**

Questions Response - 01/04 a 30/06/2015

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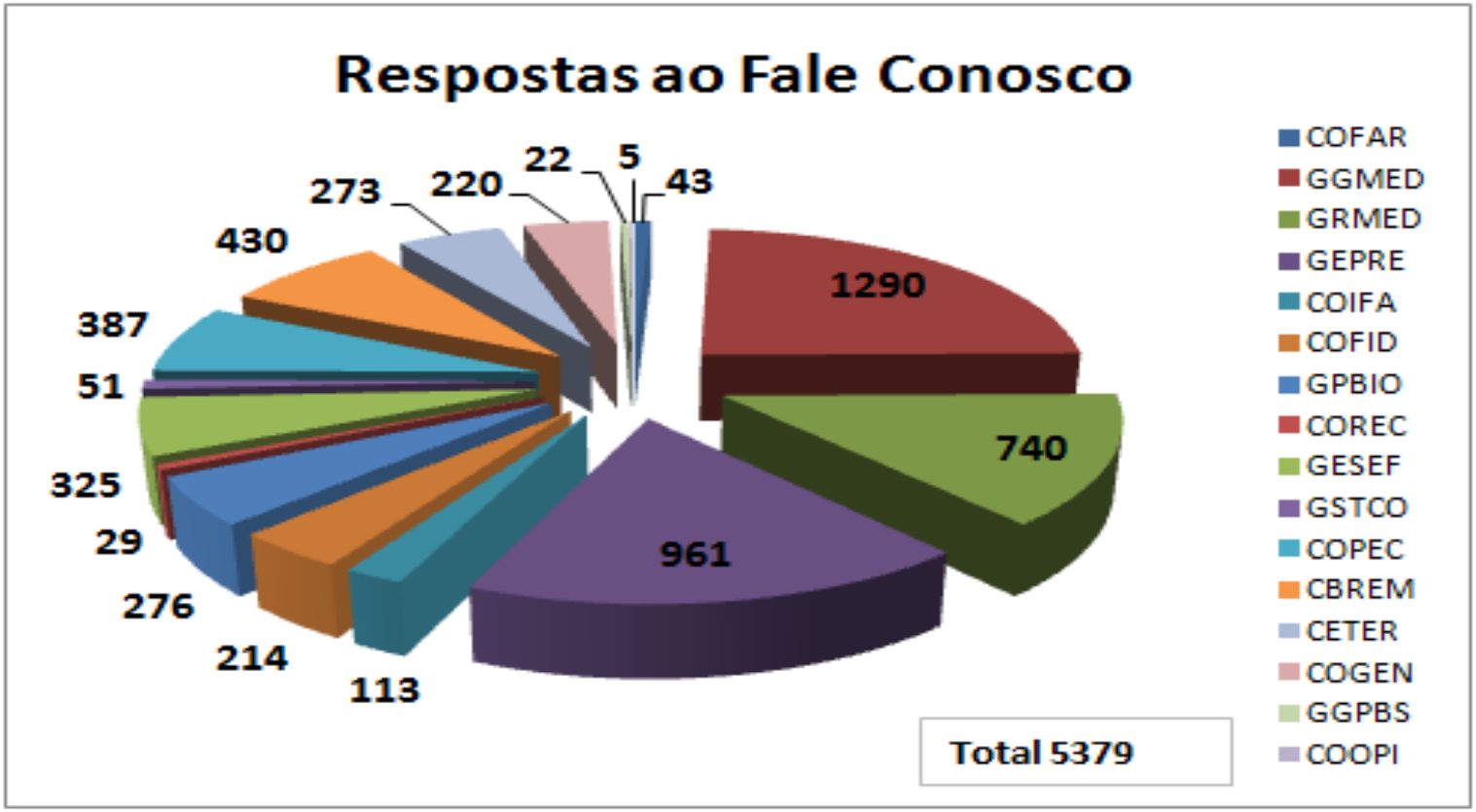

Face to face meetings 01/04 a 30/06/2015

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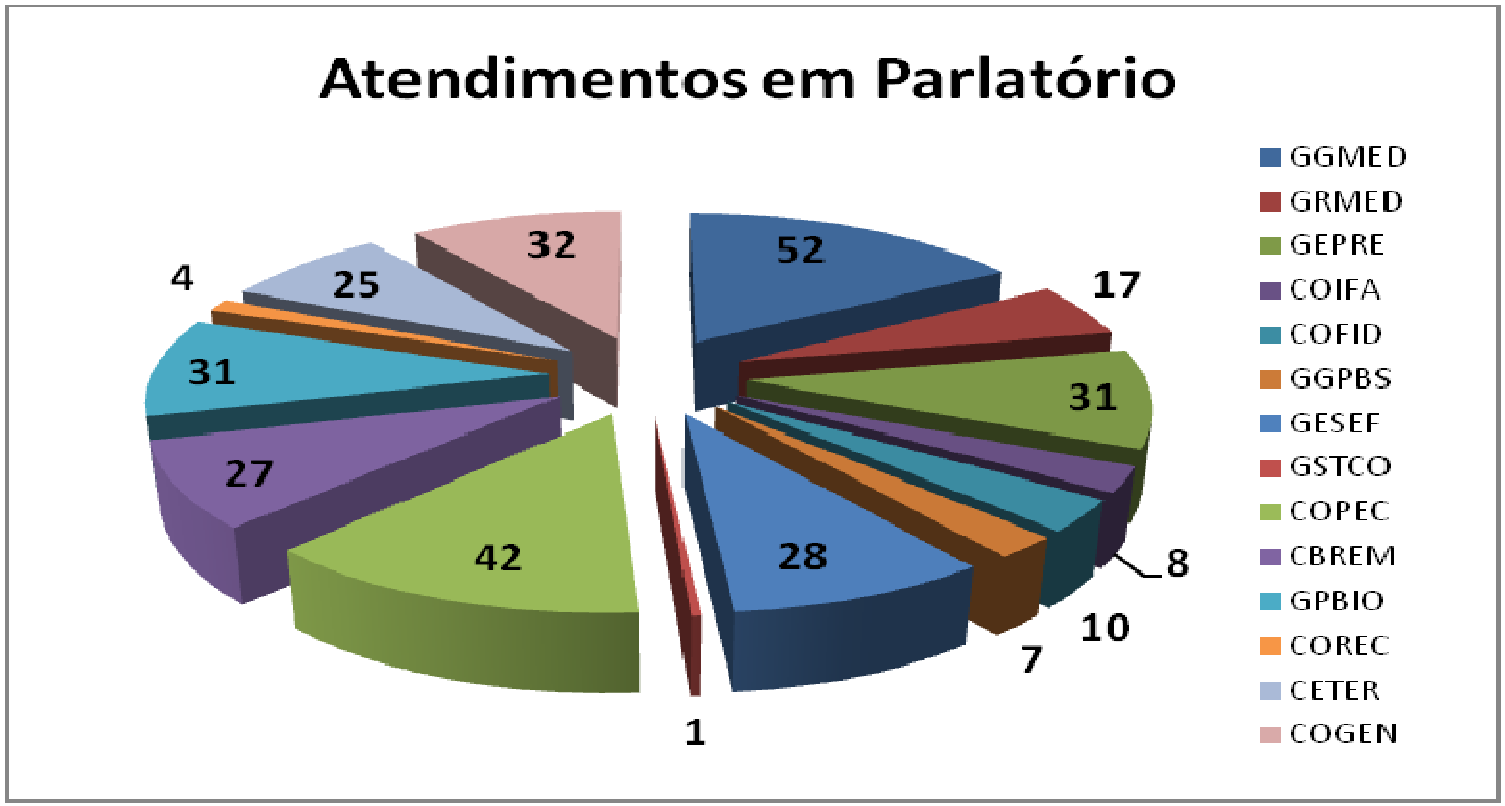

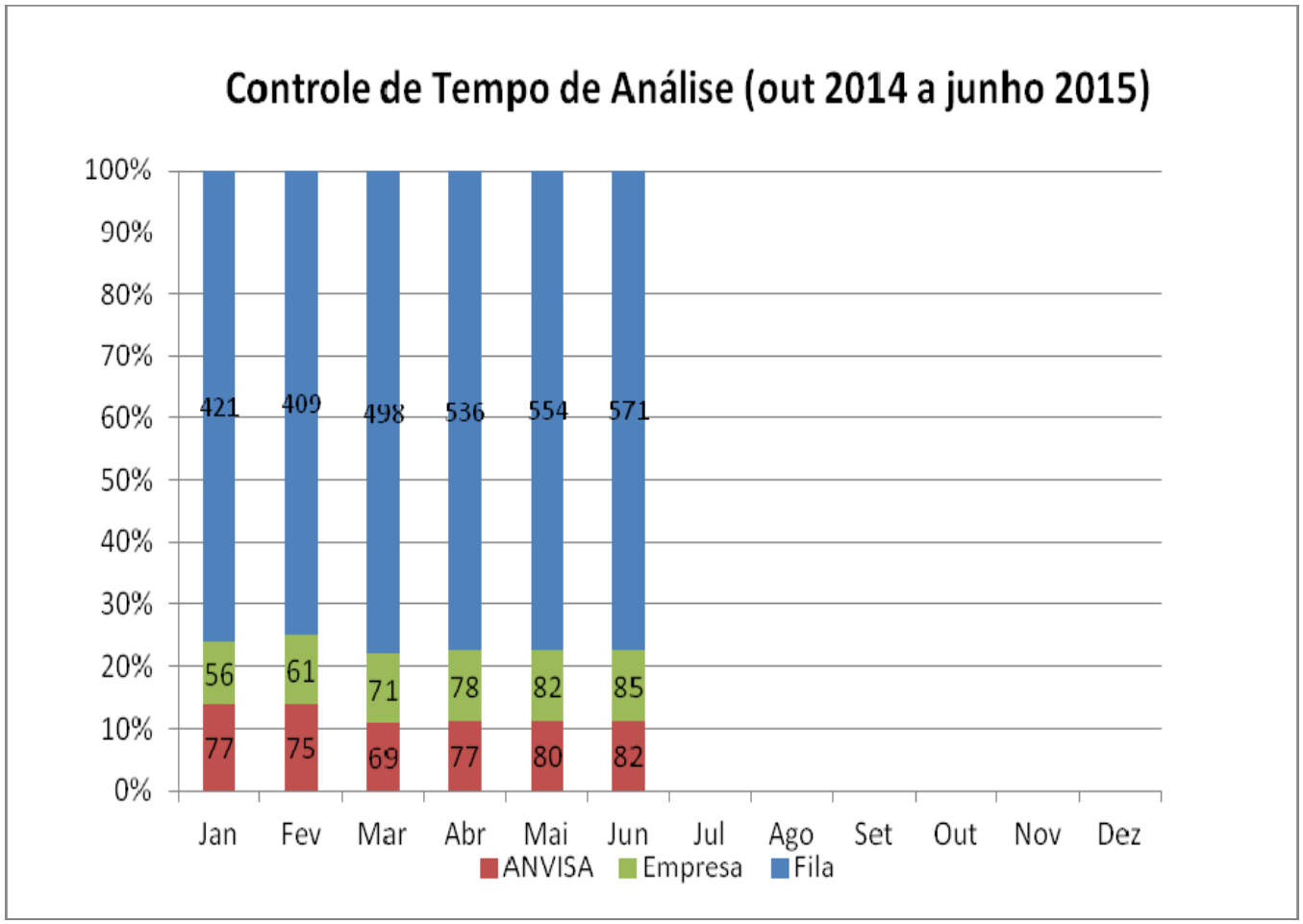
Controle de Tempo de Análise (out 2014 a junho 2015)
100%
90%
80%
70%
60%
50%
40%
30%
20%
10%
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421
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Empresa
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Line- Drugs News

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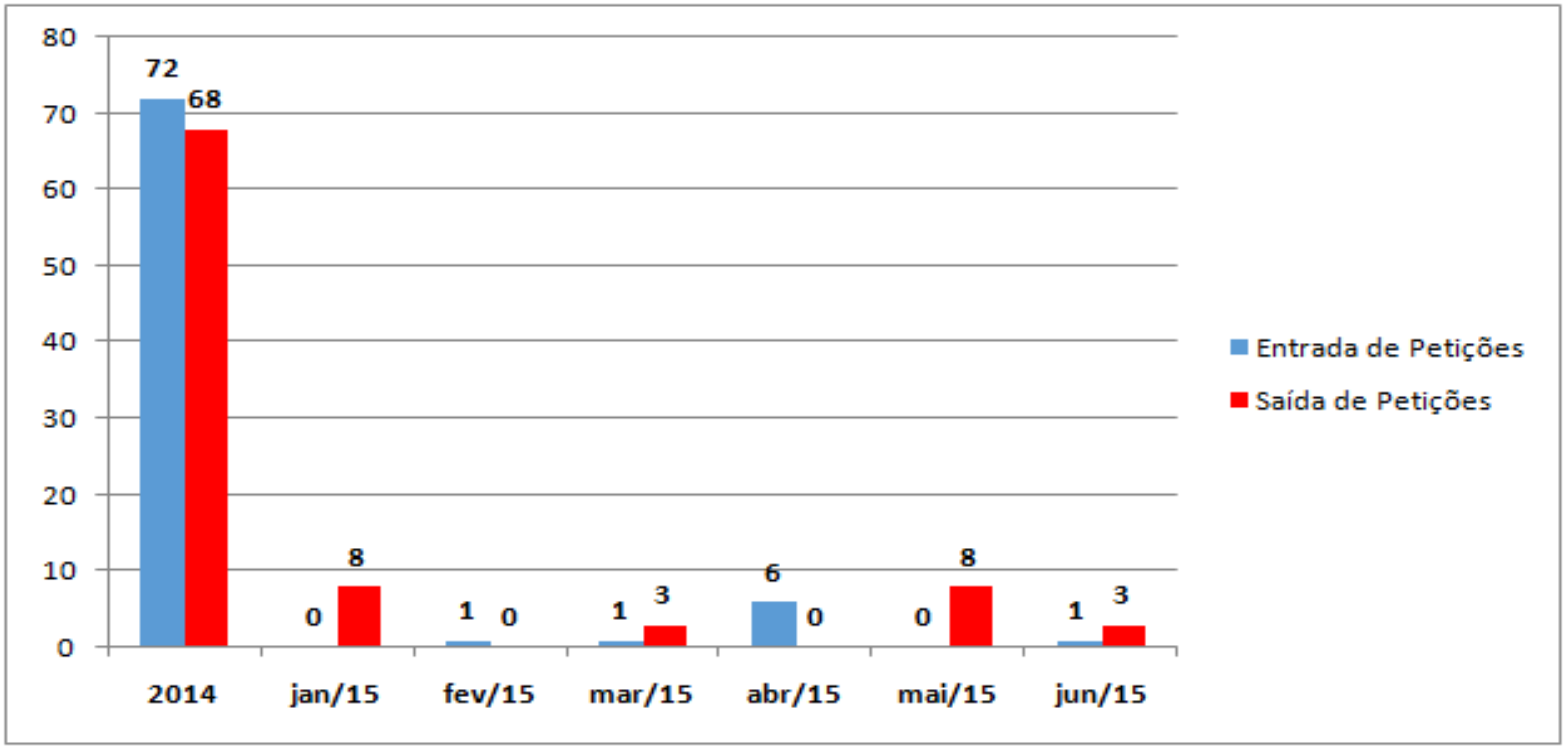

Results- Drugs News

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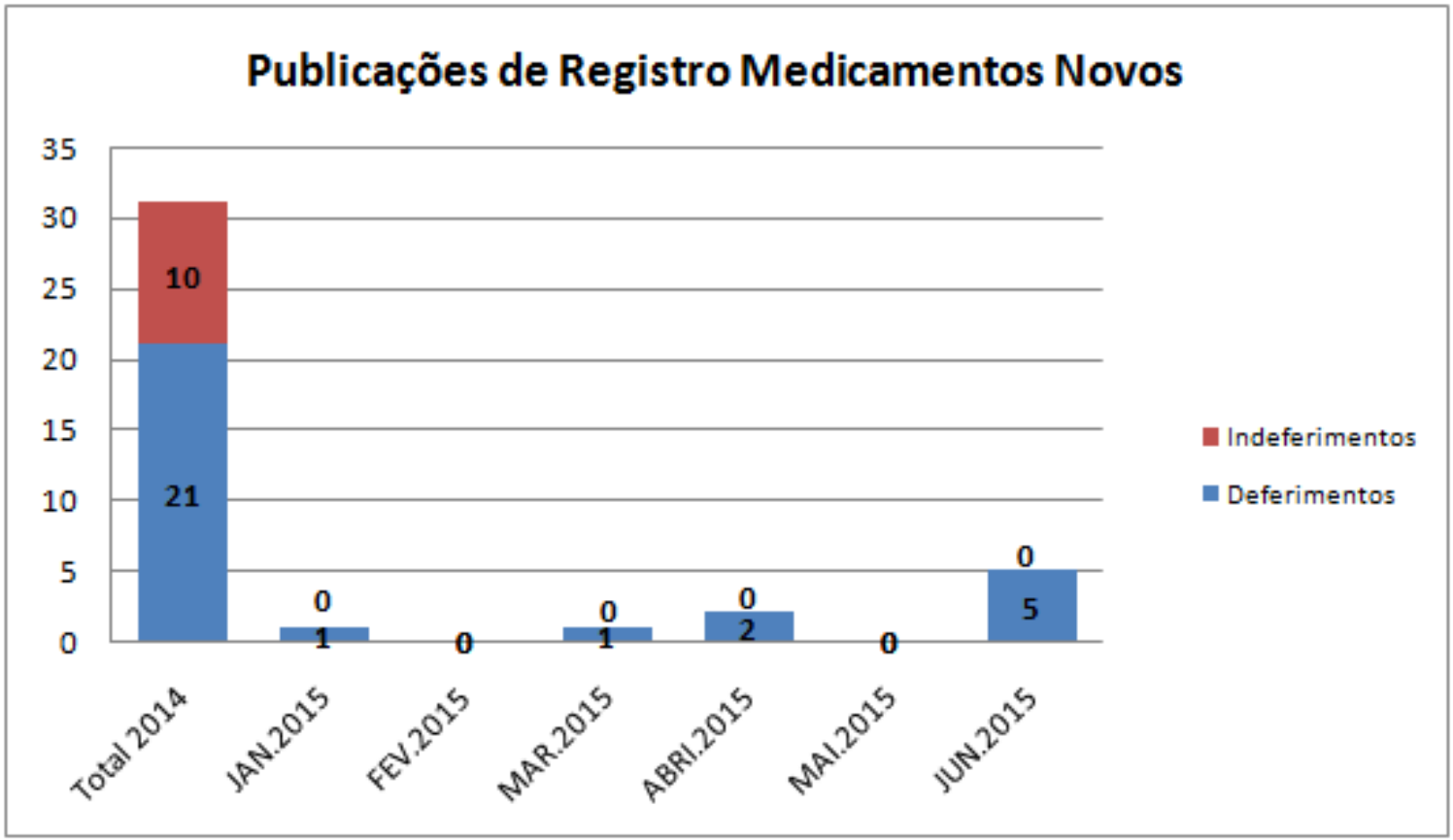

What We Want?

- Promote the development of research in the national territory and greater insertion of Brazil in the researches simultaneously conducted in different countries.

- Fully accompany the development of the product – possibility to follow the trials of priority drugs (rare diseases, unmet medical needs, etc.).

- Improve our regulatory capacity in the efficacy and safety assessment of drugs, specially those of high complexity – oncological, rare diseases.

- Maximum transparency in our actions and provide quality information on drugs to the population.

Thank you

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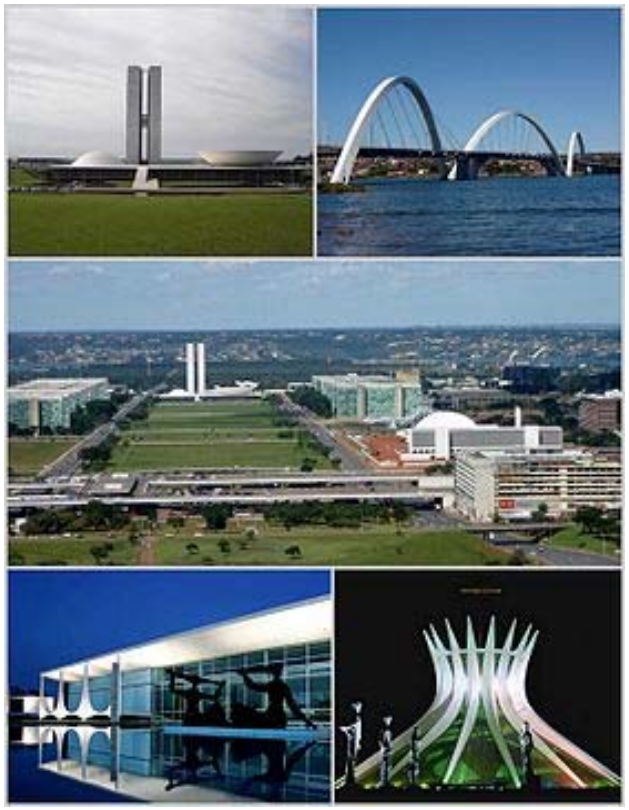

Acknowledgments

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