

## Access to treatment for phenylketonuria by judicial means in Rio Grande do Sul, Brazil

Acesso ao tratamento para fenilcetonúria por via judicial no Rio Grande do Sul, Brasil

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**Abstract** *Treatment of phenylketonuria (PKU) includes the use of a metabolic formula which should be provided free of charge by the Unified Health System (SUS). This retrospective, observational study sought to characterize judicial channels to obtain PKU treatment in Rio Grande do Sul (RS), Brazil. Lawsuits filed between 2001–2010 and having as beneficiaries PKU patients requesting treatment for the disease were included. Of 20 lawsuits filed, corresponding to 16.8% of RS patients with PKU, 19 were retrieved for analysis. Of these, only two sought to obtain therapies other than metabolic formula. In all the other 17 cases, prior treatment requests had been granted by the State Department of Health. Defendants included the State (n = 19), the Union (n = 1), and municipalities (n = 4). In 18/19 cases, the courts ruled in favor of the plaintiffs. Violation of the right to health and discontinuation of State-provided treatment were the main reasons for judicial recourse. Unlike other genetic diseases, patients with PKU seek legal remedy to obtain a product already covered by the national pharmaceutical assistance policy, suggesting that management failures are a driving factor for judicialization in Brazil.*

**Key words** *Judicialization of health, Unified Health System, Phenylketonuria*

**Resumo** *O tratamento da fenilcetonúria (PKU) inclui o uso de uma fórmula metabólica (FM) fornecida sem custos pelo Sistema Único de Saúde (SUS). O objetivo do estudo foi caracterizar o uso da via judicial para obter tratamento para PKU no estado do Rio Grande do Sul (RS), Brasil, através de um estudo retrospectivo e observacional, analisando ações judiciais. Foram incluídas ações judiciais arquivadas entre 2001–2010 que possuíam como beneficiários indivíduos com PKU solicitando alguma forma de tratamento para PKU. Foram localizados 20 casos, correspondendo a 16,8% dos pacientes com PKU no RS, sendo 19 obtidos para análise. Somente dois procuravam obter outras terapias que a FM. Nos outros 17 casos, uma solicitação de tratamento anterior fora concedida pela Secretaria Estadual de Saúde. Os réus incluem o Estado (n = 19), União (n = 1) e municípios (n = 4). Em 18/19 casos, os tribunais decidiram a favor dos demandantes. Violação do direito à saúde e interrupção do tratamento prestado pelo Estado foram os principais motivos para recorrer aos tribunais. Diferente de outras doenças genéticas, os pacientes com PKU buscam o meio jurídico para obter um produto já incluso na política de assistência farmacêutica nacional, sugerindo que falhas de gestão são um dos fatores desencadeantes da judicialização no país.*

**Palavras-chave** *Judicialização da saúde, Sistema Único de Saúde, Fenilcetonúria*

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## Introduction

Brazil has a publicly funded Unified Health System (*Sistema Único de Saúde*, SUS) that aims to provide universal and free care to the Brazilian population. The SUS pharmaceutical assistance program provides for a certain group of medicines (those that are “listed”) to be made available free of charge by the government. More than 500 medicines and supplies are currently listed, including those considered essential by the WHO (e.g., furosemide and prednisone), strategic medicines (e.g., thalidomide and zidovudine), and specialized medicines (such as olanzapine and infliximab), as well as supplies and medicines for hospital use<sup>1</sup>. Nevertheless, the use of lawsuits to obtain access to medicines is a frequent phenomenon in Brazil<sup>2</sup>. In the case of so-called rare diseases, such lawsuits, according to the literature, relate mainly to new technologies<sup>3-6</sup>.

This article uses as a study model the rare genetic disorder phenylketonuria (PKU), which has an incidence of 1:12,000-16,000 in Southern Brazil<sup>7,8</sup>. PKU is caused by deficient activity of the hepatic enzyme phenylalanine hydroxylase (PAH). This enzyme catalyzes conversion of the amino acid phenylalanine (Phe) into tyrosine, which plays an important role in the production of the neurotransmitters dopamine and norepinephrine<sup>9</sup>. As a result, patients with untreated PKU have elevated plasma concentrations of Phe, which are toxic to the central nervous system and can cause mental retardation and seizures, among other manifestations<sup>10</sup>. PKU was the first inborn error of metabolism to be treated successfully, in a landmark study by Bickel in 1953<sup>11</sup>. The treatment of PKU includes lifelong administration of specific metabolic formulas, free of Phe but rich in essential amino acids, and adherence to a low-Phe diet. Both must be adjusted on an individual basis, according to the individual tolerance of each patient and target levels of Phe for each age group. In Brazil, metabolic formulas for PKU are listed in the Specialized Program for Pharmaceutical Assistance (*Componente Especializado de Assistência Farmacêutica*, CEAF) and are thus provided free of charge in accordance with the criteria established by the Brazilian Clinical Protocol and Practice Guideline for PKU (CPPG)<sup>8</sup>. Other treatment strategies that can be used to control Phe levels are the use of special foods (for example, foods made from low-Phe flour) and supplementation with branched long-chain amino acids (which compete with Phe, preventing its absorption and

entry into the central nervous system) and BH<sub>4</sub>, a cofactor of the PAH enzyme<sup>12-15</sup>; however, SUS does not cover any of these other strategies. The diagnosis of PKU is established by measuring the concentration of Phe in whole blood samples or dried blood spot testing, ideally in a neonatal screening framework, because early treatment, provided regularly and without interruption, prevents the development of mental retardation and other neurological complications<sup>16,17</sup>. No studies have reported on the use of legal recourse to obtain access to PKU treatment strategies in Brazil. Our hypothesis is that patients have difficulty accessing these treatments in the country due to failures in implementation of existing public policies.

## Methods

This retrospective, observational study, approved by the local Research Ethics Committees, sought to characterize the use of legal recourse to obtain treatment for PKU in the State of Rio Grande do Sul (RS), Brazil. Located in the southernmost region of the country, RS has a population of 11,164,043, a birth rate of 11.6 per 1,000, and a per capita gross domestic product (GDP) of R\$ 23,606.00<sup>18</sup>. The state has two public referral centers specializing in monitoring patients with PKU, at Hospital de Clínicas de Porto Alegre (HCPA) and Hospital Materno Infantil Presidente Vargas (HMIPV), both of which are public hospitals located in the capital city of Porto Alegre. At the start of data collection (2011), there were 119 known patients with PKU being monitored at these centers. Of these, 62 were treated at HCPA and 57 at HMIPV. The 2001–2010 period was chosen in an attempt to standardize data, as, in 2001, the Brazilian National Neonatal Screening Program (which includes the diagnosis of PKU) was established, and, in 2002, the first CPPG for this disease was published<sup>19</sup> (with an update in 2010<sup>8</sup>). Within this context, we identified and analyzed all lawsuits filed between 2001 and 2010 in which beneficiaries were patients with PKU who sought some form of treatment for this disease. For each lawsuit identified, variables for the period elapsed from filing of the lawsuit to the court ruling were analyzed.

Of the 119 patients followed by the two reference centers in RS, 114 patients had an indication for use of Phe-free formula (e.g., they had Phe levels at diagnosis  $\geq 6$  mg/dL; patients who have Phe levels at diagnosis between 2 and 6 mg/

dL have hyperphenylalaninemia but do not require treatment), and 20 of these 114 (17.5%) had secured access to the formula by means of litigation. In RS, to request treatment by administrative means, a patient diagnosed with PKU must go to the Municipal Health Department of his or her municipality of residence with a prescription for treatment. This request undergoes technical evaluation by a healthcare professional assigned by the State and, when appropriate, the prescribed medicine is authorized for subsequent dispensation. Upon receipt of approval, the medicine is dispensed to the patient, usually once monthly; therefore, requests for treatment must be reevaluated on a quarterly basis.

The initial data about the 20 lawsuits (including lawsuit number, procedural framework, etc.) were obtained from the electronic databases of the state and federal justice systems<sup>20,21</sup>. After this step, the authors contacted (by email, telephone, or in person) the notaries of the counties involved and the subsections of the Brazilian Bar Association (*Ordem dos Advogados do Brasil*, OAB) at each municipality, and requested full-text copies of the legal proceedings. Using this strategy, the files of 11 cases were obtained. The remaining nine cases could not be obtained due to a refusal or delay in meeting the requests for copies by county notaries (n = 3) or because the lawsuits involved children or adolescents (n = 6). An official letter was then delivered to the Internal Affairs Department of the State Court of Justice requesting access to these case files for research purposes. This strategy yielded copies of eight additional lawsuits (one request was overruled). Thus, the study sample includes 19 cases, all up to date as of the end of 2011.

Two specific forms (available upon request) were prepared by the authors for collection of data from case files. The process of creating the forms is described elsewhere by Leivas e Schwartz<sup>22</sup>.

The first form was more extensive, including items designed to collect information about the processing of lawsuits and on the arguments advanced by the lawyers or plaintiffs in support of the filing, the defense, injunctions, blocks on government accounts, judgments, and appeals. The arguments were classified into legal, empirical (medical/research/economic), or related to administrative issues. For example, legal arguments included references to children's rights and the right to health; medical arguments included references to evidence-based medicine (scientific papers, clinical reports, and clinical guidelines re-

lated to PKU); economic arguments mentioned aspects of cost-effectiveness, scarcity of resources, lack of budgetary provisions, and impact on public budgets; and arguments related to administrative issues included references to difficulties that the State of RS faces in controlling its stocks of PKU metabolic formula and difficulties involving procurement processes.

The second form was designed for examination of questions about the plaintiffs and defendants, such as profile of the lawsuit beneficiary, source of and rationale for the prescription, type of medicine, and manner of request therefor. Prescriptions were considered adequate when they were in agreement with the CPPG for PKU published in 2002<sup>19</sup>. To enable proper evaluation of prescriptions, knowledge of the age and weight of the patients and their current plasma Phe levels was necessary.

Data collection was done by two legal professionals (Forms 1 and 2) and one health professional (Form 2), who subsequently met to reach a consensus instrument for each lawsuit, and whose data were entered into a database and analyzed as means and frequencies. All variables for which information was available from at least 70% of the sample were analyzed.

## Results

Considering the 19 lawsuits included in the study, the average time elapsed between date of filing until 12/31/2011 was 2,117 days (approximately 6 years). The first lawsuit was filed on 01/05/2001, and the last, on 08/20/2010. The average time elapsed between filing and ruling was 648 days (approximately 1.5 years). In 17 cases, there was a report of prior administrative approval of request for PKU treatment; in the two remaining cases, this information was not available. All analyzed cases filed documentation confirming the diagnosis of PKU (prescriptions, medical reports, or statements).

Table 1 contains data on the profile of the beneficiaries of the lawsuits and the defendants, as well as on the claims made in the initial petitions. Data on the filings are shown in Table 2. According to medical reports attached to the lawsuits, the most common justification for medical prescription was the risk of developing neurological problems associated with non-treatment of PKU.

Figure 1 details the claims most frequently made by the plaintiffs in the initial application.

**Table 1.** Access to Phenylketonuria treatment by judicial means in Rio Grande do Sul, Brazil: characterization of the sample (n = 19 lawsuits).

Variables	N
Patient variables	
Gender (M:F)	12:7
Age	
0-2 years	4
3-11 years	12
12-18 years	1
≥ 19 years	1
N/A	1
Lawsuit variables	
Defendant	
State of Rio Grande do Sul	14
State of Rio Grande do Sul + Municipality	4
Union + State of Rio Grande do Sul	1
Author/plaintiff	
Public defender	8 (42.1)
Public prosecutor	7 (36.8)
Private attorney	4 (21.1)
Request for phenylketonuria formula	
Yes	18 (94.7)
No*	1 (5.3)
Request for special foods and/or drugs	
Yes**	2 (10.5)
No	17 (89.5)

\* Request referred solely to special foods for PKU. \*\* One patient requested metabolic formula and anticonvulsant medication. The other patient requested infant formula for the first year of life and PKU formula. N/A = not available.

**Table 2.** Access to Phenylketonuria treatment by judicial means in Rio Grande do Sul, Brazil: summary of clinical information available in lawsuits (n = 19 cases).

Variables	N
Medical prescription attached to the lawsuit	18/19
Medical report attached to the lawsuit	14/19
Treatment prescribed	
Phenylketonuria formula	17/18
Special foods*	1/18
Others**	4/18
Patient weight reported in lawsuit	
Yes	3/19
No	16/19
Adequate medical prescription	
Yes	3/18
Could not be determined	15/18

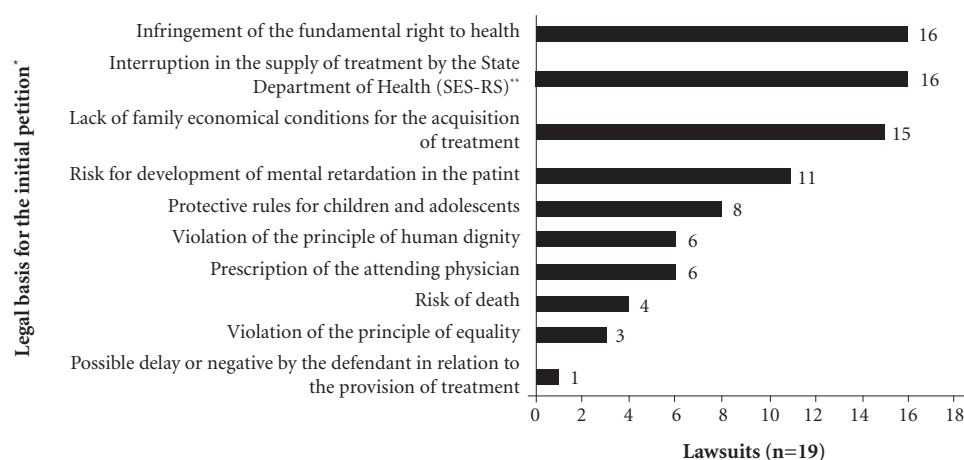
\* Low-Phe pasta and flour. \*\* In three lawsuits, the beneficiaries had been prescribed infant formula for the first year of life as well as PKU formula; in one lawsuit, the beneficiary had been prescribed anticonvulsant medication and PKU formula.

Table 3 shows that all requests for advance relief were granted. Regarding the manner in which orders were fulfilled, in all cases, the judge determined that the defendant acquire the necessary supplies for treatment of the applicants; in one lawsuit, the defendant was given the option of paying the cost of treatment to the applicant in cash. Table 4 provides detailed information on the grounds for ruling in each lawsuit.

Overall, 18 of the 19 lawsuits were contested: 15 by the state government alone, two by the state and municipal governments, and one by the federal and state governments.

Regarding the arguments used by the defense, in 12 of 18 cases, the defendant advanced arguments related to medical aspects. The most common medically based arguments were: discussions on replacement of formula provided at concentrations different from those approved at the administrative level (n = 6/12); need for submission of medical reports stating that the patient requires treatment (n = 1/12); one claim that the treatment was not included in the SUS list of supplies to be provided free of charge (claim filed prior to the 2002 CPPG) (n = 1/12); and lack of up-to-date medical reports (n = 8/12). In five cases (n = 5/18), the defendants argued economic aspects, such as lack of budgetary provision for the acquisition of the requested treatment, as well as also scarce resources (n = 1/5); impact on the public budget (n = 2/5); impact on the public budget, scarce resources, and the principle of the proviso of possibility (n = 1/5); and resources insufficient to comply with the decision (n = 1/5). In three of the 18 cases, defendants cited administrative problems related to difficulties in inventory control and delays in procurement processes as reasons for the non-availability of the formula. Regarding the legal and constitutional aspects mentioned in the disputes, the most frequent discussions and complaints concerned the fundamental right to health (n = 6/10), the principle of reserve for contingencies (n = 4/10), and the illegitimacy of the lawsuit being filed by the public prosecutor (n = 4/10). These were followed by violation of the principle of equality (n = 3/10), administrative discretion/principle of separation (n = 3/10), violation of the principle of human dignity (n = 1/10), and others (n = 1/10).

In 13 cases (n = 13/19), bank accounts of the State of RS were frozen. The sum of these assets was R\$ 228,112.39 (range = R\$ 1,831.32 to R\$ 52,313.38). On average, two account freezes were ordered during each lawsuit; seven cases led to two freezes each, and one lawsuit involved six account freezes.



**Figure 1.** Access to PKU treatment by judicial means in Rio Grande do Sul, Brazil: legal basis for the initial petition.

\* Medical and research-related, economic, legal, and constitutional issues found most frequently in the arguments during analysis of initial petitions. \*\* State Department of Health of Rio Grande do Sul, Brazil (Secretaria Estadual de Saúde, SES-RS).

## Discussion

Despite the large number of policies and public lawsuits involving existing pharmaceutical assistance programs in Brazil, the practice of recourse to the courts as a means of securing supply of medicines through the SUS has provoked discussions regarding implementation of the right to health. This is a markedly Brazilian phenomenon, whereby lawsuits requesting access to medicines via the SUS are brought before the federal and state courts as a faster and more effective alternative both to obtain access to new health technologies and to ensure access to medicines that should be supplied free of charge by the SUS<sup>23-25</sup>.

According to previous research conducted by our group, using the genetic diseases mucopolysaccharidoses and Fabry disease as models, the main cause for judicialization in the field of rare diseases in Brazil is the search for access to technologies not yet incorporated into the SUS<sup>4-6</sup>.

In the cases studied herein, most recipients previously requested and were granted treatment by extrajudicial (administrative) means, in compliance with the criteria established in the Clinical Protocol and Practice Guideline for PKU<sup>8</sup>. A priori, resorting to the judicial system to obtain access to the PKU formula seems contradictory, but the data we obtained suggest that

interruptions in the supply of the formula, violations of the fundamental right to health, and the economic conditions of the families of PKU patients, limiting their ability to pay for treatment out of pocket, are the key factors that led these patients to seek legal remedies to ensure access to PKU treatment. It bears stressing that, during the study period, there was no evidence of shortages of metabolic formula due to insufficient manufacturing and/or distribution by the pharmaceutical industry.

Analysis of the defendants in the analyzed lawsuits revealed that the State of RS was a defendant in all lawsuits assessed. This was already expected by the authors, as the plaintiffs had previously been granted access to treatment through the SUS via administrative means.

The limited information contained in the lawsuits precluded a more in-depth characterization of the profile of the plaintiffs. Unfortunately, data on income, occupation, and education were available only in few cases. Other data suggest that the beneficiaries of the studied cases were mostly children. The predominance of male subjects is likely due to chance, as PKU is an autosomal recessive disease and a gender difference in prevalence is not expected.

Recently, empirical studies about the judicialization of health have sought to advance the argument that this phenomenon is being taken up

**Table 3.** Access to Phenylketonuria treatment by judicial means in Rio Grande do Sul, Brazil: data on advance relief (n = 19).

Variables	N
Manner of compliance with court decision	
Defendant ordered to provide medicines	18
Defendant ordered to provide medicine and/or financial resources for the patient to purchase treatment	1
Periodicity of provision of the formula as set by the court	
Once monthly (enough for one month)	18
Twice yearly (enough for one semester)	1
Arguments used in ruling <sup>*</sup>	
Related to medical or research aspects	
Availability of medical reports	12
Availability of evidence <sup>**</sup>	7
Risk of death/serious harm to life associated with non-treatment	1
Risk of developing mental retardation associated with non-treatment	1
Related to legal and constitutional aspects	
Violation of the right to health	12
Violation of children's rights	6
Violation of the principle of human dignity	1
Violation of the principle of priority	1
Related to economic aspects <sup>***</sup>	0
Related to administrative issues <sup>****</sup>	0

<sup>\*</sup> More than one argument may have been cited in the ruling. <sup>\*\*</sup> References to scientific articles, medical reports, and clinical protocols related to phenylketonuria were considered as evidence. <sup>\*\*\*</sup> References to cost-effectiveness of the requested treatment, scarcity of resources for the acquisition of supplies, lack of budgetary provisions for the procurement of supplies, and impact on the public budget were considered economic aspects. <sup>\*\*\*\*</sup> References to difficulties that the State of RS faces in controlling its inventory of PKU metabolic formula and difficulties with procurement processes were considered administrative aspects.

by economic elites, which goes against the constitutional principles of the SUS, such as equity. The upholding of this complex theory depends on a specific methodology being able to obtain evidence contributing to the characterization of the problem; however, said evidence remains insufficient<sup>26</sup>. Our study found several indications that contradict this thesis of the elites. In our sample, lawsuits were filed mainly by public defenders and by the Office of the Public Prosecutor; few lawsuits were filed by private attorneys. Data such as family income and occupation were not available in statistically significant quantities to enable proper characterization of the plaintiffs. All prescriptions originated from public university hos-

**Table 4.** Access to Phenylketonuria treatment by judicial means in Rio Grande do Sul, Brazil: data on court rulings (n = 18).

Variables	N
Time elapsed between lawsuit filing and ruling	
< 6 months	5
6 months to 1 year	3
1 to 2 years	6
> 2 years	4
Ruling	
Upheld <sup>*</sup>	17
Partially upheld <sup>**</sup>	1
Denied <sup>*</sup>	1
Arguments used in the ruling <sup>‡</sup>	
Related to medical and research aspects	
Availability of medical reports	13
Availability of evidence <sup>§</sup>	9
Availability of treatment alternatives	1
Related to legal and constitutional aspects	
Violation of the right to health	15
Violation of children's rights	6
Violation of the principle of human dignity	4
Reserve for contingencies/principle of proportionality	1
Administrative discretion/separation of powers	1
Violation of the principle of equality	1
Illegitimacy	1
Others <sup>  </sup>	3
Related to administrative issues <sup>¶</sup>	0

<sup>\*</sup> In one lawsuit, the ruling was upheld against the State of RS and dismissed against the County, which was cleared of the responsibility to provide PKU formula. <sup>\*\*</sup> In this case, the ruling determined that three cans of PKU formula be supplied, whereas the lawsuit requested four cans. <sup>‡</sup> More than one argument may have been cited in the ruling. <sup>§</sup> References to scientific articles, medical reports, and clinical protocols related to phenylketonuria were considered as evidence. <sup>||</sup> One lawsuit discussed the legitimacy of prosecutors to plead individual rights; the other addressed the absence of a challenge to the plaintiff's claim by the State of RS; the third discussed the right to life. <sup>¶</sup> References to difficulties that the State of RS faces in controlling its inventory of PKU metabolic formula and difficulties with procurement processes were considered administrative aspects.

pitals, which is not a good indicator of social class, as these facilities house referral services in patient care, research, and technology in the field of genetics, as well as providing equitable and universal access through the SUS. Hence, we cannot say whether the judicialization of PKU treatment in RS is being driven by economic elites or whether it is correlated with socioeconomic status.

A request for PKU formula was made in almost all lawsuits; in one case, there was no re-

quest for the formula, but rather for special foods. The attempt to obtain special foods for PKU management via judicial means may have been prompted by the fact that patients or their families do not have sufficient financial resources or access to these foods, which, in the case of PKU, are imported and expensive. However, cultural issues and the difficulty of families to cope with a chronic disease that may not be socially acceptable could also be associated with poor adherence to a diet based on these special foods, prioritizing the use of judicial means to obtain the metabolic formula. In general, patients seek legal recourse to obtain medicines. However, it bears stressing that, for the treatment of PKU, access to a diet that enables control of plasma Phe levels is as important as access to the PKU formula, and combined administration of these treatment strategies enables the PKU patient to achieve acceptable Phe concentrations.

Regarding prescriptions, most beneficiaries of the lawsuits had already had prior administrative applications for the metabolic formula accepted by the State Department of Health (SES-RS), which suggests that these patients had a proper prescription for the formula. However, due to the study methodology, analysis of prescribing data for PKU metabolic formula could not determine whether the amount of formula requested or the prescription for formula were appropriate. Information on plasma Phe levels (both at the time of diagnosis and current) and patient weight, which are needed to calculate the proper dose of metabolic formula, were usually missing. Data on which type of formula was prescribed were often missing as well.

The role of the Public Prosecutor as a procedural proxy of the beneficiary was often challenged by the defendants, generating extensive discussions about the legitimacy of the prosecution in litigating for individual interests through civil action. The ability of the Public Prosecutor to take upon himself the role of guarantor of unavailable individual interests related to health issues, as well as protection of the Statute for Children and Adolescents, is settled in jurisprudence of the Superior Court of Justice (STJ)<sup>27</sup>.

As expected, the most frequent “economic” arguments of the defense were related to the lack of budgetary provisions for the implementation of judicial decisions, the impact of these decisions on the public budget, and the violation of constitutional principles such as the proviso of possibility. The right to health was also discussed in the arguments of the defense, but only regard-

ing the recognition by the State of its responsibility to provide adequate pharmaceutical services to citizens. In some lawsuits, the State of RS admitted its own problems and difficulties in managing inventory of PKU formula due to delays in procurement processes. These facts strongly indicate a possible reason for the interruptions in supply of the PKU formula by the State of RS, which motivated these adversely affected patients to seek access to the formula by judicial means.

Another fact that deserves attention concerns medical and research-related arguments presented by the State in legal defenses. In defense strategies, the State sought to discuss applications for replacement of treatment with PKU formula at concentrations different from those approved by the State of RS at the administrative level, requesting that the patient present evidence proving the need for treatment through medical reports; requesting updated reports; and claiming that the formula was not included in any list of medicines required to be provided by the State. Importantly, the beneficiaries of the lawsuits had already had administrative requests for treatment previously approved; as stated earlier, treatment of PKU is lifelong and consists of the administration of a specific metabolic formula, which, in Brazil, is listed in the CEAF formulary and is distributed free of charge by the State of RS in accordance with the CPPG criteria. The State demonstrated a lack of technical knowledge about PKU and about its own policies, perhaps because of miscommunication between the various sectors involved.

Analysis of advance relief is of utmost importance for understanding the phenomenon of judicialization of health in Brazil. In the cases examined, all beneficiaries had their applications for advance relief granted. As a general rule, the granting of injunctions was based on the facts alleged in the medical reports and the precepts of the right to health. No injunction mentioned the existence of a CPPG for PKU. This corroborates the findings of other studies on judicialization of health, in which judges were found to rule in favor of supplying medicines without compliance with current SUS policies for pharmaceutical care<sup>28</sup>.

It is also important to realize that injunctive relief requires that the State of RS provide the PKU formula as requested in the initial petitions made by the beneficiaries, i.e., in the quantities and type of formula prescribed and requested. Failure to observe these decisions led to garnishment of State accounts. We found that, within the

time frame of the study, most requests for freezing of government accounts were granted. On one hand, decisions to garnish in government accounts assure immediate compliance of judicial orders for applicants, but these same decisions may have a negative impact on the performance of the State, as they prevent the State from exercising its negotiating flexibility or bargaining power through procurement processes that meet budgetary provisions. This may expose the health care system to a rise in costs corresponding to emergency purchases that bypass standard procurement processes<sup>5,29</sup>.

At the end of the study period, almost all lawsuits had been upheld at final decision, confirming access to the formula and ensuring the individual right to health of each applicant as previously signaled by the granting of injunctions. The underlying aspects of the final rulings were similar to those used as grounds for injunctive relief, *i.e.*, focusing on medical aspects and on the right to health.

Our data were reported to the RS State Department of Health, and some initiatives are

underway in an attempt to overturn judicialization of PKU treatment, including the possibility of direct dispensation of the formula at referral centers to newly diagnosed patients. However, we believe it is essential that the State develop better inventory control strategies so as to avoid shortages of medication if a tender is impugned, among other possibilities.

## Conclusions

Our data reveal that discontinuation of supply of the PKU formula is the main cause of judicialization of PKU treatment in RS, suggesting breakdowns in the management of pharmaceutical services in the State, and that the right to health is the main legal foundation for legal decisions favorable to patients/plaintiffs. Therefore, requests for access to health technologies not yet incorporated into the SUS are not always the leading cause of judicialization of rare disease treatment in Brazil.



## Collaborations

LM Trevisan conceived the study, collected, analyzed and interpreted the data and wrote the paper; T Nalin and T Tonon conceived the study, collected the data and wrote the paper; LM Veiga collected and analyzed the data and wrote the paper; P Vargas interpreted the data and wrote the paper; BC Krug collected, analyzed and interpreted the data and wrote the paper; PGC Leivas conceived the study, collected, analyzed and interpreted the data and wrote the paper; IVD Schwartz conceived the study, analyzed and interpreted the data and wrote the paper. Approval of final version for publication: all authors approved the final version for publication.

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## References

1. Brasil. Ministério da Saúde (MS). Secretaria de Ciência, Tecnologia e Insumos Estratégicos. Departamento de Assistência Farmacêutica e Insumos Estratégicos. *Relação Nacional de Medicamentos Essenciais (RENAME)* [Internet]. Brasília: Editora MS; 2010. [cited 2013 Dec 5]. Available from: [http://bvsm.s.saude.gov.br/bvs/publicacoes/renome\\_2010.pdf](http://bvsm.s.saude.gov.br/bvs/publicacoes/renome_2010.pdf)
2. Biehl J, Amon JJ, Social MP, Petryna A. Between the court and the clinic: lawsuits for medicines and the right to health in Brazil. *Health Hum Rights* 2012; 14(1):E36-52.
3. Travassos DV, Ferreira RC, Vargas AM, de Moura RN, Conceição EM, Marques DF, Ferreira EF. The judicialization of health care: a case study of three state courts in Brazil. *Cien Saude Colet* 2013; 18(11):3419-3429.
4. Sartori Junior D, Leivas PG, Souza MV, Krug BC, Balbinotto G, Schwartz IV. Court-ordered access to treatment of rare genetic diseases: Fabry Disease in the state of Rio Grande do Sul, Brazil. *Cien Saude Colet* 2012; 17(10):2717-2728.
5. Diniz D, Medeiros M, Schwartz IV. Consequences of the judicialization of health policies: the cost of medicines for mucopolysaccharidosis. *Cad Saude Publica* 2012; 28(3):479-489.
6. Boy R, Schwartz IV, Krug BC, Santana-da-Silva LC, Steiner CE, Acosta AX, Ribeiro EM, Galera MF, Leivas PG, Braz M. Ethical issues related to the access to orphan drugs in Brazil: the case of mucopolysaccharidosis type I. *J Med Ethics* 2011; 37(4):233-239.
7. Jardim LB, Palma-Dias R, Silva LC, Ashton-Prolla P, Giugliani R. Maternal hyperphenylalaninaemia as a cause of microcephaly and mental retardation. *Acta Paediatr* 1996; 85(8):943-946.
8. Brasil. Ministério da Saúde (MS). *Protocolo Clínico e Diretrizes Terapêuticas - Fenilcetonúria* [Internet]. Brasília: MS; 2010. [cited 2013 Dec 5]. Available from: [http://portal.saude.gov.br/portal/arquivos/pdf/pcdt\\_fenilcetonuria.pdf](http://portal.saude.gov.br/portal/arquivos/pdf/pcdt_fenilcetonuria.pdf)
9. Olsson GM, Montgomery SM, Alm J. Family conditions and dietary control in phenylketonuria. *J Inherit Metab Dis* 2007; 30(5):708-715.
10. Mitchell JJ, Trakadis YJ, Scriver CR. Phenylalanine hydroxylase deficiency. *Genet Med* 2011; 13(8):697-707.
11. Zschocke J, Hoffmann GF. *Vademecum Metabolicum: Manual of Metabolic Paediatrics*. 2<sup>nd</sup> ed. Friedrichskoog: Schattauer; 2004.
12. Blau N. *PKU and BH4: Advances in Phenylketonuria and Tetrahydrobiopterin*. Chennai: Scientific Publishing Services; 2006.
13. Trefz FK, Blau N. Potential role of tetrahydrobiopterin in the treatment of maternal phenylketonuria. *Pediatrics* 2003; 112(6 Pt 2):1566-1569.
14. Perez-Duenas B, Vilaseca Vilaseca MA, Mas A, Lambruschini N, Artuch R, Gómez L, Pineda J, Gutiérrez A, Mila M, Campistol J. Tetrahydrobiopterin responsiveness in patients with phenylketonuria. *Clin Biochem* 2004; 37(12):1083-1090.
15. Karam SM. *Avaliação epidemiológica da triagem neonatal para fenilcetonúria no Rio Grande do Sul - 1986-2003: um estudo de coorte* [tese]. Porto Alegre: Universidade Federal do Rio Grande do Sul; 2004.

16. Brasil. Ministério da Saúde (MS). Secretaria de Assistência à Saúde. Coordenação-Geral de Atenção Especializada. *Manual de Normas Técnicas e Rotinas Operacionais do Programa Nacional de Triagem Neonatal* [Internet]. Brasília: Editora MS; 2002. [cited 2013 Dec 05]. Available from: [http://bvms.saude.gov.br/bvs/publicacoes/triagem\\_neonatal.pdf](http://bvms.saude.gov.br/bvs/publicacoes/triagem_neonatal.pdf)
17. Souza CFM, Schwartz IV, Giugliani R. Neonatal screening of metabolic disorders. *Cien Saude Colet* 2002; 7(1):129-137.
18. Instituto Brasileiro de Geografia e Estatística (IBGE). *Censo Demográfico - Rio Grande do Sul* [Internet]. Rio de Janeiro: IBGE; 2010. [cited 2013 Dec 5]. Available from: <http://www.ibge.gov.br/estadosat/perfil.php?sigla=rs>
19. Brasil. Ministério da Saúde (MS). *Protocolo Clínico e Diretrizes Terapêuticas - Fenilcetonúria* [Internet]. [cited 2013 Dec 5]. Brasília: MS; 2002. Available from: [http://dtr2001.saude.gov.br/sas/dsra/protocolos/do\\_f20\\_01.pdf](http://dtr2001.saude.gov.br/sas/dsra/protocolos/do_f20_01.pdf)
20. Brasil. Tribunal de Justiça do Estado do Rio Grande do Sul (TJRS). *Acompanhamento Processual* [Internet]. 2011 [cited 2013 Dec 5]. Available from: <http://www.tjrs.jus.br/site/>
21. Brasil. Tribunal Regional Federal da 4ª Região (TRF). *Consulta Processual - Rio Grande do Sul* [Internet]. 2011 [cited 2013 Dec 5]. Available from: <http://www2.trf4.jus.br/trf4/>
22. Leivas PGC, Schwartz IVD. A construção do instrumento de avaliação de processos judiciais em pesquisas interdisciplinares sobre as causas da “judicialização da saúde”. VIII SEPesq – Semana de Pesquisa, Pós-graduação e Extensão do Centro Universitário Ritter dos Reis; Porto Alegre: Ritter dos Reis; 2012.
23. Rios RR. *Direito à saúde, universalidade, integralidade e políticas públicas: princípios e requisitos em demandas judiciais por medicamentos* [Internet]. Porto Alegre: Revista de Doutrina da 4ª Região; 2009. [cited 2013 Dec 5]. Available from: [http://www.revistadoutrina.trf4.jus.br/index.htm?http://www.revistadoutrina.trf4.jus.br/artigos/edicao031/roger\\_rios.html](http://www.revistadoutrina.trf4.jus.br/index.htm?http://www.revistadoutrina.trf4.jus.br/artigos/edicao031/roger_rios.html)
24. Pepe VL, Ventura M, Sant’ana JM, Figueiredo TA, Souza Vdos R, Simas L, Osorio-de-Castro CG. Characterization of lawsuits for the supply of “essential” medicines in the State of Rio de Janeiro, Brazil. *Cad Saude Publica* 2010; 26(3):461-471.
25. Biehl J, Petryna A, Gertner A, Amon JJ, Picon PD. Judicialisation of the right to health in Brazil. *Lancet* 2009; 373(9682):2182-2184.
26. Medeiros M, Diniz D, Schwartz IV. The thesis of judicialization of health care by the elites: medication for mucopolysaccharidosis. *Cien Saude Colet* 2013; 18(4):1079-1088.
27. Brazil. Superior Tribunal de Justiça (STJ). *Recurso Especial 716.512 - RS (2005/0004911-6)* [Internet]. Brasília: Superior Tribunal de Justiça; 2005. [cited 2013 Dec 05]. Available from: <http://stj.jusbrasil.com.br/jurisprudencia/7185655/recurso-especial-re-sp-716512-rs-2005-0004911-6/inteiro-teor-12923833>
28. Chieffi AL, Barata RB. ‘Judicialization’ of public health policy for distribution of medicines. *Cad Saude Publica* 2009; 25(8):1839-1849.
29. Pandolfo M, Delduque MC, Amaral RG. Legal and sanitary aspects conditioning access to medicines in Brazilian courts. *Rev Salud Publica (Bogota)* 2012; 14(2):340-349.

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