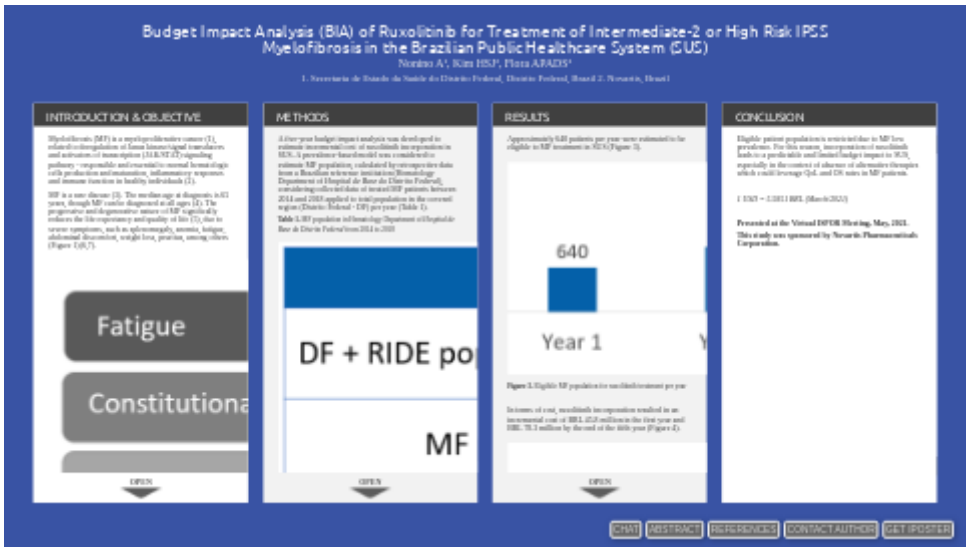
Budget Impact Analysis (BIA) of Ruxolitinib for Treatment of Intermediate-2 or High Risk IPSS Myelofibrosis in the Brazilian Public Healthcare System (SUS)



Nonino A[1], Kim HSJ[2], Flora APADS[2]

1. Secretaria de Estado da Saúde do Distrito Federal, Distrito Federal, Brazil 2. Novartis, Brazil

PRESENTED AT:

INTRODUCTION & OBJECTIVE

Myelofibrosis (MF) is a myeloproliferative cancer (1), related to deregulation of Janus kinase/signal transducers and activators of transcription (JAK/STAT) signaling pathway - responsible and essential to normal hematologic cells production and maturation, inflammatory responses and immune function in healthy individuals (2).

MF is a rare disease (3). The median age at diagnosis is 65 years, though MF can be diagnosed at all ages (4). The progressive and degenerative nature of MF significally reduces the life expectancy and quality of life (5), due to severe symptoms, such as splenomegaly, anemia, fatigue, abdominal discomfort, weight loss, pruritus, among others (Figure 1) (6,7).

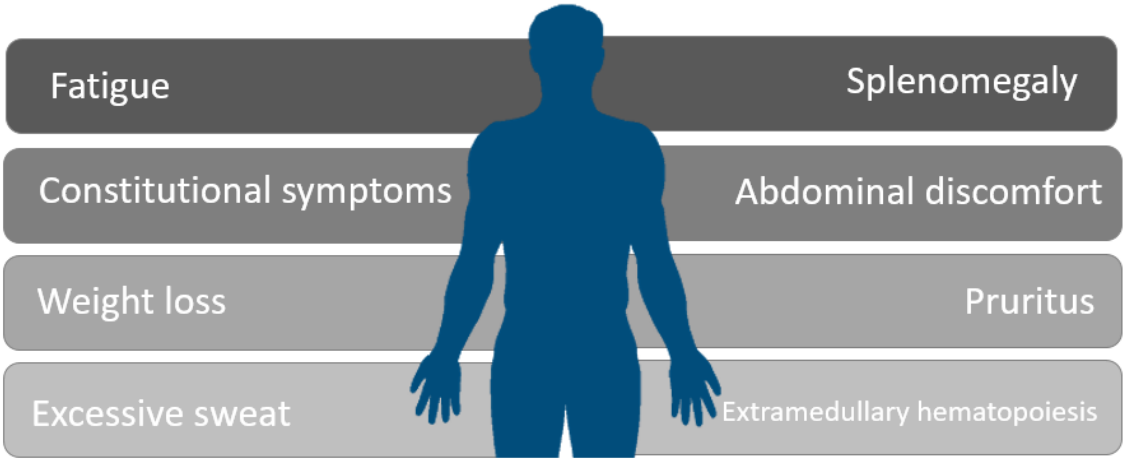


Figure 1. MF main symptoms

Even though hematopoietic stem cell transplantation (HSCT) is the only curative therapeutic procedure, most of MF patients are ineligible to HSCT due to advanced age, presence of comorbidities, or donor unavailability(8). In cases of ineligibility to HSCT, pharmacological therapies considered as best available therapy (BAT), such as hydroxyurea, danazol and alfa-interferon are the therapeutic alternatives indicated for these patients. However, there are limited data demonstrating clinical benefits of BAT, especially in terms of overall survival (OS) and quality of life (QoL) improvements in MF population(9).

Considering this context, ruxolitinib is a selective and potent JAK1 and JAK2 inhibitor. It is the first approved drug in Brazil for MF treatment(10), based on clinical benefits, including OS and QoL, demonstrated in phase III clinical trials of ruxolitinib versus placebo or BAT(11,12,13).

OBJECTIVE

To evaluate the budget impact of ruxolitinib incorporation for patients with primary or secondary MF, classified as intermediate-2 or high risk, according to the International Prognostic Scoring System (IPSS), and ineligible for HSCT in the Brazilian public healthcare system (SUS).

METHODS

A five-year budget impact analysis was developed to estimate incremental cost of ruxolitinib incorporation in SUS. A prevalence-based model was considered to estimate MF population, calculated by retrospective data from a Brazilian reference institution (Hematology Department of *Hospital de Base do Distrito Federal*), considering collected data of treated MF patients between 2014 and 2018 applied to total population in the covered region (Distrito Federal - DF) per year (Table 1).

Table 1. MF population in Hematology Department of *Hospital de Base do Distrito Federal* from 2014 to 2018

	2014	2015	2016	2017	2018
DF + RIDE population	4118154	4204866	4291577	4341733	4560505
MF patients	23	28	37	33	27
Prevalence per 100,000 inhabitants	0.56	0.67	0.86	0.76	0.59

DF: *Distrito Federal*; RIDE: covered DF region (*Região integrada de desenvolvimento do Distrito Federal e entorno*)

Final prevalence calculated from the 5-year data was 0.69 MF cases per 100,000 inhabitants per year.

In order to obtain the total MF cases in Brazil, the resulting DF prevalence of 0.69/100,000 inhabitants was extrapolated to total Brazilian population projection for the next five years (14). Additionally, from the total MF population, 49% of patients were assumed to have intermediate-2 and high risk IPSS (5), and 11% were assumed to be ineligible to ruxolitinib due to platelet counts lower than 50x10^9/L (15) (Figure 2).

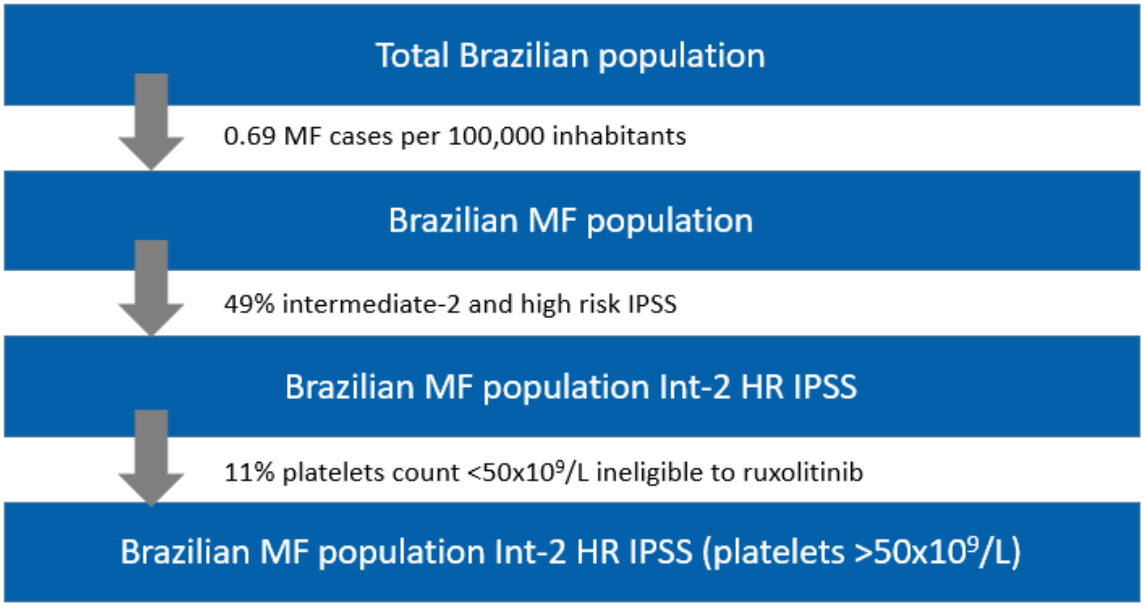


Figure 2. Population rational flow

For the scenario without ruxolitinib, 100% of patients were assumed to use reimbursement procedures for MF therapy in SUS. Market-share of ruxolitinib inclusion ranged from 30% (year 1) to 50% (year 5).

Cost of treatment consisted of official Brazilian maximum government sales price (16) and DATASUS database (17) (Table 2). For the comparator (APAC) cost, a weighted average was calculated, considering procedure costs in SUS and respective usage in 2018/2019, extracted from DATASUS database (17).

Table 2. Monthly costs considered in the model

	Monthly cost	Reference
Ruxolitinib (15 mg/20 mg)	BRL 20,273.18	CMED price list[16]
APAC*	BRL 399.70	DATASUS database[17]

*APAC 1L (BRL 150) – 85% usage; APAC 2L (BRL 1,800) – 15% usage in 2018/2019

RESULTS

Approximately 640 patients per year were estimated to be eligible to MF treatment in SUS (Figure 3).

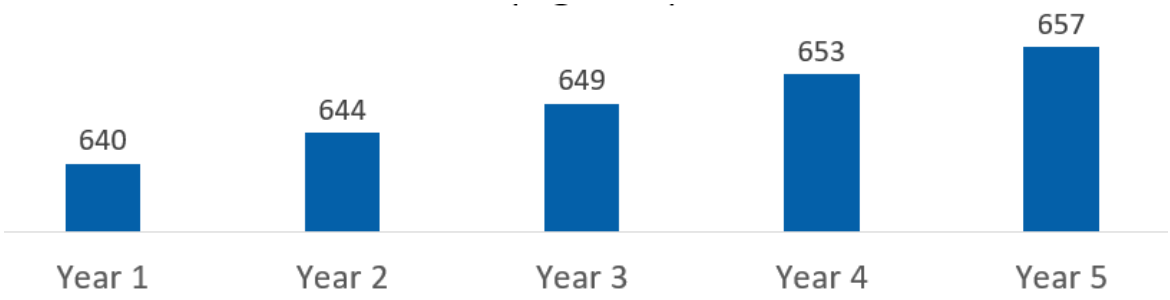


Figure 3. Eligibile MF population for ruxolitinib treatment per year

In terms of cost, ruxolitinib incorporation resulted in an incremental cost of BRL 45.8 million in the first year and BRL 78.3 million by the end of the fifth year (Figure 4).

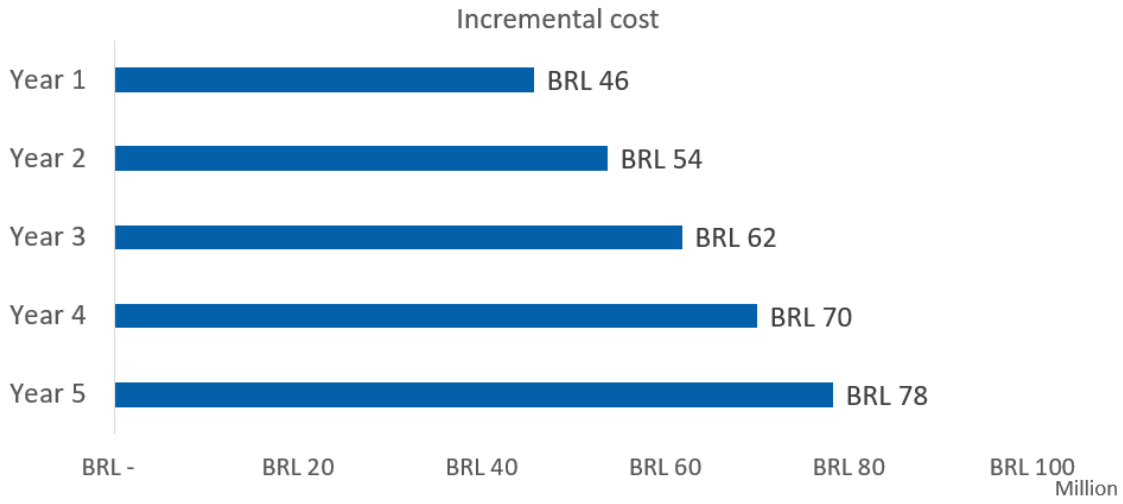


Figure 4. Incremental costs of ruxolitinib incorporation in SUS per year

CONCLUSION

Eligible patient population is restricted due to MF low prevalence. For this reason, incorporation of ruxolitinib leads to a predictable and limited budget impact to SUS, especially in the context of absence of alternative therapies which could leverage QoL and OS rates in MF patients.

1 USD = 5.5851 BRL (March/2021)

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ABSTRACT

OBJECTIVES

To evaluate the budget impact of ruxolitinib incorporation for patients with primary or secondary myelofibrosis (MF), classifed as intermediate-2 or high risk, according to the International Prognostic Scoring System (IPSS), and ineligible for hematopoietic stem cell transplantation in the Brazilian publichealthcare system (SUS).

METHODS

A five-year BIA was developed to estimate incremental cost of ruxolitinib incorporation in SUS. A prevalence-based model was considered to estimate MF population, calculated by retrospective data from a Brazilian reference institution (Hematology Department of *Hospital de Base do Distrito Federal*), considering collected data of treated MF patients between 2014 and 2018 applied to total population in the covered region (Distrito Federal - DF) per year. For extrapolation to total Brazilian population, the resulting DF prevalence of 0.69/100,000 inhabitants was applied to population projection in Brazil for the next five years. Additionally, 49% patients were assumed to have intermediate-2 and high risk IPSSc(Cervantes, 2009), and 11% were assumed to be ineligible to ruxolitinib due to platelet counts lower than 50x10^9/L (Devos, 2015). For the scenario without ruxolitinib, 100% of patients were assumed to use reimbursement procedures for MF therapy in SUS. Market-share of ruxolitinib inclusion ranged from 30% (year 1) to 50% (year 5). Cost of treatment consisted of oficial Brazilian maximum government sales price and DATASUS database.

RESULTS

Approximately 640 patients per year were estimated to be eligible to MF treatment in SUS. In terms of cost, ruxolitinib incorporation resulted in an incremental cost of BRL45.78 million in the first year and BRL78.34 million by the end of the fifth year.

CONCLUSIONS

Eligible patient population is restricted due to MF low prevalence. For this reason, incorporation of ruxolitinib leads to a predictable and limited budget impact to SUS, especially in the context of absence of alternative therapies with benefits in quality of life and overall survival.

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