Patient Perspectives

2023 ISPOR Transparency in health technology assessment: How much is too much?

GenerAtion patient

Generation patient

Events

HAS
HEALTH ADVOCACY SUMMIT

The Health Advocacy Summit is the prior name of the overall organization but now serves as the name for our virtual and in-person events. Prior to the pandemic, we facilitated seven in-person Summits in four states and during the pandemic, we have facilitated three international virtual Summits.

Programs

CCYAN
Crohn's and Colitis Young Adults Network

The Crohn's and Colitis Young Adults Network (CCYAN) facilitates a fellowship program, community space, and more for young adults with Inflammatory Bowel Diseases. CCYAN is the only disease-specific programming of Generation Patient. Visit www.ccyanetwork.org.

(HEALTH) policy lab

The mission of the (Health) Policy Lab is to provide health policy education and advocacy opportunities to young adults with chronic and rare conditions in an effort to increase meaningful access to prescription medications. Visit www.hplab.org.

& More!

- 6 Virtual Meetings per month
- Roundtables to bring together stakeholders on a variety of topics
- Advocacy to increase access to higher education
- Critical resources for our community, including civic engagement and advance care planning.

Disclosures

Our support comes from foundations such as the Helmsley Charitable Trust, Arnold Ventures, the Commonwealth Fund, the Disability Inclusion Fund, Third Wave Fund, and the Lumina Foundation.

We are independent of all private healthcare industry funding.

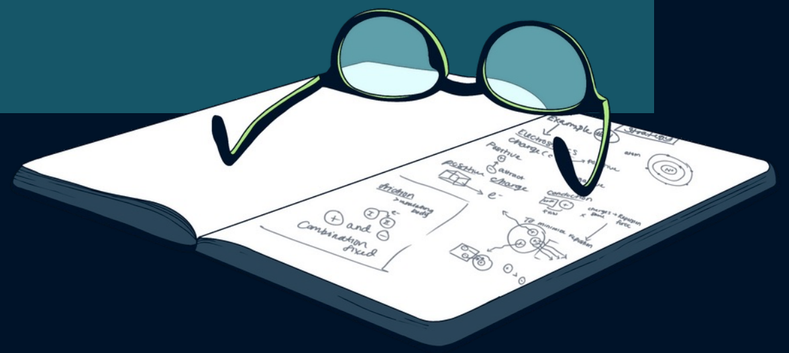
Patient Demographics

Why is it important for patients to have access to clinical trial data?

Informed decision-making: Having access to information about the efficacy and safety of different treatments can provide an understanding of the risks and benefits of the treatment.

For many patients:

- Lack of time
- Lack of updated, evidence-based guidelines for the condition
- Lack of localized quality of care, medical harm, and medical trauma.

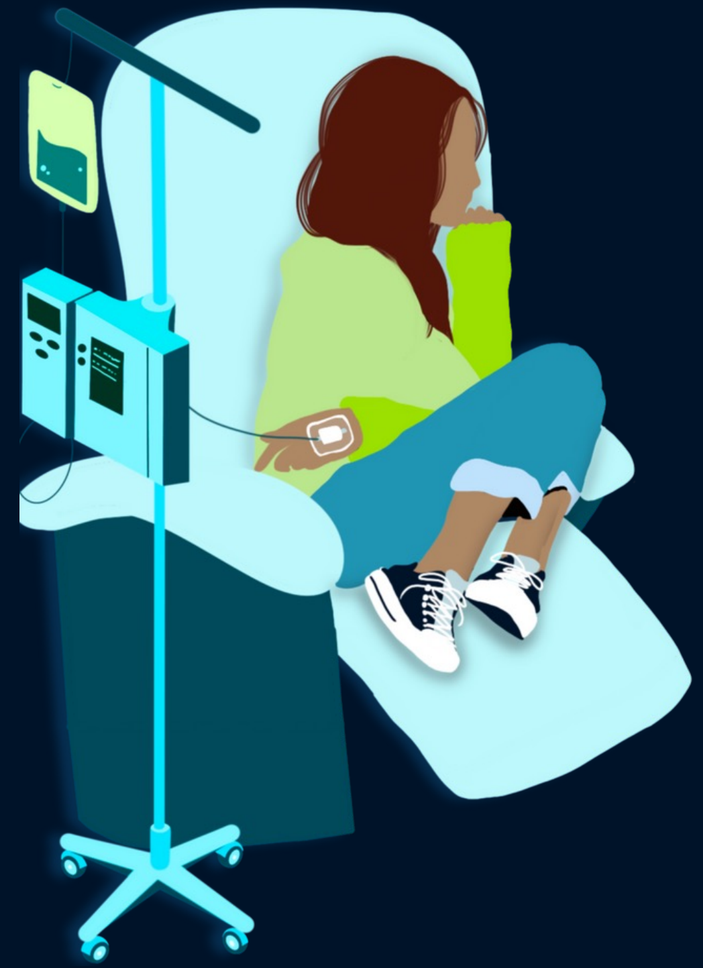


Patient Perspectives

- Short and long-term safety
  - Not to just avoid harm, but help patients manage side effects
- Short and long-term efficacy
- Redacting information can cause inconsistency in the understanding other information
- Affordability of therapeutics
- Subgroup analysis: “Were patients like me included?”

Patient Perspectives

- If I am going to be paying for this therapeutic, directly or indirectly, should I not have access to all information about it to make an informed decision?
- Competition of therapeutics versus single therapeutic market
- For patients especially on multiple therapeutics, knowing adverse events is critical.
  - Which drug is more likely to cause the particular side effect?



Examples of redaction

Clinical Review
Laura Jawidzik, MD
NDA [redacted] and 212728
Rimegepant [redacted]

Table 48 Distribution of Patients by Maximum Number of Attacks Treated in One Month

Maximum number of attacks per month	75 mg N=1798 n(%)
0	4 (0.2)
>0 and <2	17 (0.9)
≥2 and <4	105 (5.8)
≥4 and <6	257 (14.3)
≥6 and <8	318 (17.7)
≥8 and <10	303 (16.9)
≥10 and <12	241 (13.4)
≥12 and <14	151 (8.4)
≥14	402 (22.4)

This table was adapted from an IR response dated November 13, 2019 eCTD seq no 0019.

Reviewer's comment: The applicant has proposed that dosing of rimegepant should be labeled 75 mg [redacted] The product is intended for chronic intermittent usage, not [redacted] as this statement implies. [redacted]

Given the concern for medication overuse (MO) in patients using acute migraine medications, it would be prudent to limit the use of rimegepant to a frequency below which MO is known to develop. For triptans this threshold is around 10 migraines per months and for NSAIDs this threshold is considered to be around 15 days per month. I recommend a statement in the label, "The safety of treating more than 15 migraines a month has not been established."

Screenshot of rimegepant FDA multi-disciplinary review.

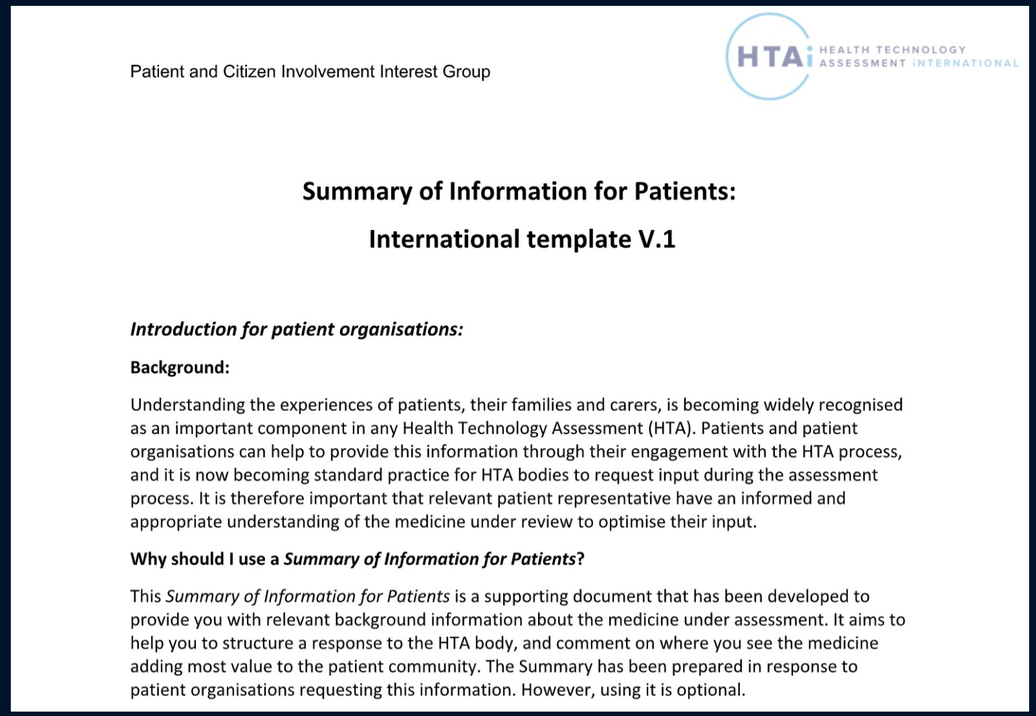
Incidence, n (%)	Group 1: PRN 2-8[a] (N=1,033)	Group 2: PRN 9-14[a] (N=481)	Group 3: Scheduled EOD + PRN[a] (N=286)	Overall (N=1,800)
SAE	[redacted]	[redacted]	[redacted]	[redacted]
SAE related to study drug[b]	[redacted]	[redacted]	[redacted]	[redacted]
AE leading to study drug discontinuation	24 (2.3)	16 (3.3)	8 (2.8)	48 (2.7)
Hepatic-related AE	[redacted]	[redacted]	[redacted]	[redacted]
Severe hepatic-related AE	[redacted]	[redacted]	[redacted]	[redacted]
Hepatic-related SAE	[redacted]	[redacted]	[redacted]	[redacted]
Hepatic-related AE leading to study drug discontinuation	[redacted]	[redacted]	[redacted]	[redacted]
AEs associated with potential abuse	[redacted]	[redacted]	[redacted]	[redacted]
Cardiovascular AE	[redacted]	[redacted]	[redacted]	[redacted]
Suicidality AE	[redacted]	[redacted]	[redacted]	[redacted]
AEs reported in ≥2% overall				
Upper respiratory tract infection	[redacted]	[redacted]	[redacted]	[redacted]
Nasopharyngitis	[redacted]	[redacted]	[redacted]	[redacted]
Sinusitis	[redacted]	[redacted]	[redacted]	[redacted]
Urinary tract infection	[redacted]	[redacted]	[redacted]	[redacted]
Influenza	[redacted]	[redacted]	[redacted]	[redacted]
Back pain	[redacted]	[redacted]	[redacted]	[redacted]
Bronchitis	[redacted]	[redacted]	[redacted]	[redacted]
Nausea	[redacted]	[redacted]	[redacted]	[redacted]
Dizziness	[redacted]	[redacted]	[redacted]	[redacted]
Arthralgia	[redacted]	[redacted]	[redacted]	[redacted]

Abbreviations: AE, adverse event; EOD+ PRN, scheduled EOD+PRN, every other day dosing plus as needed on nonscheduled dosing days for up to 12-weeks; PRN, *pro re nata* (as needed) dosing; SAE, serious adverse event
Notes:
[a]Treatment groups were as follows: (1) PRN 2-8: historical rate of 2-8 moderate to severe migraine attacks per month with dosing as needed (PRN) up to a maximum of 1 tablet of rimegepant 75 mg per day; (2) PRN 9-14: historical rate of 9-14 migraine attacks per month with dosing as needed (PRN) up to a maximum of 1 tablet of rimegepant 75 mg per day; (3) Scheduled EOD dosing with as needed (PRN) dosing to treat a migraine attack of any severity on non-schedule days
[b]Events were considered related to treatment if the relationship was not reported or rated as unlikely related, possibly related or related per investigator assessment.
Refereces: Data on File Clinical Study Report BHV3000-201; [140] Croop 2020b [222]

Screenshot of rimegepant NICE review (p. 130).

Recommendations

- Greater awareness of multi-disciplinary reviews and assessments
- Plain language summaries for patients and healthcare providers
- Timing of information for patients
- Patients should be engaged in determining what information is critical for patient understanding
- Opportunity to link full assessments and information in electronic health records for easier access to increase equitable access to scientific material



Patient and Citizen Involvement Interest Group

HTAi HEALTH TECHNOLOGY ASSESSMENT INTERNATIONAL

Summary of Information for Patients:

International template V.1

Introduction for patient organisations:

Background:

Understanding the experiences of patients, their families and carers, is becoming widely recognised as an important component in any Health Technology Assessment (HTA). Patients and patient organisations can help to provide this information through their engagement with the HTA process, and it is now becoming standard practice for HTA bodies to request input during the assessment process. It is therefore important that relevant patient representative have an informed and appropriate understanding of the medicine under review to optimise their input.

Why should I use a *Summary of Information for Patients*?

This *Summary of Information for Patients* is a supporting document that has been developed to provide you with relevant background information about the medicine under assessment. It aims to help you to structure a response to the HTA body, and comment on where you see the medicine adding most value to the patient community. The Summary has been prepared in response to patient organisations requesting this information. However, using it is optional.

Provided by Ann Single, from the HTAi Patient and Citizen Involvement Interest Group

Contact:

sneha@generationpatient.org

@genpatient

@generationpatient

generationpatient.org