HAUTE AUTORITÉ DE SANTÉ

QUESTIONNAIRE

Patient Association Contribution Questionnaire

Evaluation of a drug for reimbursement and/or early access authorization

Validated by CEESP and CT on Nov 8 and 9 22

Sommaire

Introduction

The HAS Transparency Commission is specifically tasked with rendering opinions:

– for reimbursement upon request from drug manufacturers. These “for reimbursement” opinions, whether favorable or unfavorable, are sent to the Ministry of Health, which must decide on them.

– regarding requests for early access authorization, also at the request of manufacturers, for immediate and full management of a product that is not yet reimbursed. It is on the basis of this opinion that the HAS College issues the final decision.

The HAS has all the medical-scientific data, but cannot on its own give the arguments for reflection from the perspective of the patients or users concerned.

That is why you are being asked to contribute to the assessments.

The Commission for Economic Evaluation and Public Health issues an economic opinion on some of these drugs. This contribution will be systematically sent to it.

‘The Patient Engagement’ team is always available for any questions or exchanges. Contact: contact.contribution@has-sante.fr / 01 55 93 71 18.

All associations or groups representing patients in the health system may use this questionnaire.

You can consult the published information intended for associations and patients and users on the dedicated website of the HAS website.

In the first few times, it may be useful to receive explanations: there is always a dedicated contact person to respond to you, refer you or communicate with you by email (contact.contribution@has-sante.fr) or by telephone (01 55 93 71 18). It is the Patient Engagement department that will answer you: it is a team that listens to you.

For detailed information on the principles of drug evaluation by the Transparency Commission, you can consult the assessment doctrines for reimbursement and for early access authorization. This specialized reading is not required to complete this questionnaire.

Note: to simplify reading, the masculine is used in the sentences of this questionnaire – this is simply a writing agreement in this document. HAS promotes inclusion and equality in all aspects, including gender and gender.

Important Notes

The questions in this form are not binding. They aim to guide you if you find them relevant.

If desired, you can only use the “Other” frames that can be expanded as needed.

Only questions from the administrative part at the very beginning of this questionnaire are mandatory

Administrative information (contact, funding) for the HAS.

Identity of the association or group

Full name (followed by acronym if applicable):

Dr. Christina Tischer, Fondation européenne pour les soins aux nouveau-nés

Dr. Christina Tischer, European Foundation for the Care if Newborn Infants (EFCNI)

Internet Site :

www.efcni.org

Mailing Address (if applicable) :

Christina.Tischer@efcni.org

Nature of structure:

☐ Nationally Accredited Association

☐ Regionally accredited association

☐ Unlicensed Association

☒ Other (specify):

Organisation de parents (ONG), Munich (Bavière), Allemagne, Fondation de droit public allemand

Parent organisation (NGO), Munich (Bavaria), Germany, Foundation under German Public Law

If applicable, membership in a federation or network:

Response box (you can enlarge).

Drug on which this contribution relates

Trade name:
Nirsevimab/Beyfortus

International Common Name (ICD):
Response box (you can enlarge).

Indication for drug evaluation for reimbursement or early access authorization:
Response box (you can enlarge).

This contribution relates to an assessment (check the corresponding box or both if applicable):
As a reminder, this information appears next to the name of the medicinal product on the page dedicated to contributions on the HAS website.

☒ With a view to reimbursement under common law

☐ For Early Access Authorization Request

1. Questionnaire – Part A

1.1. Impacts of the disease on quality of life

1.1.1. Impacts of the disease on patients

You can address the topics below if they are relevant to you, and add as many aspects or parameters to consider

You can prioritize themes, you can rate their severity using a scale from 1 (less severe) to 5 (most severe)

You can detail impacts by separating 'groups', e.g. male/female, child/adult, etc.:

– Intellectual or physical fatigue

– Activities of daily living

– Mobility/travel

– In children: impact on growth and psychomotor development – Education, sports and play activities, etc.

– Work life – Ability to work

– Emotional life

– Sexual life

– Social life

– Psychological impacts

– Pain

- Financial aspects

– Other aspects

What are the impacts of the disease on patients?
Patients: Qualité de vie des nourrissons et symptômes liés à l'infection par le virus respiratoire syncytial (VRS)

Patients: infant quality of life and symptoms related to the Respiratory Syncytial Virus (RSV) infection

Are you aware of any quality of life data collection questionnaires or other patient-reported measures that you feel are appropriate for this disease?
☐ No

☒ Yes, which ones?

ResQ Family projet (ResQ Family: Impact de l'hospitalisation pour le virus respiratoire syncytial (VRS) sur la qualité de vie des familles - une étude multi-pays)

ResQ Family project (ResQ Family: Impact of Respiratory Syncytial Virus (RSV) Hospitalisation on Quality of Life of Families – A Multi-Country Study)

1.1.2. Impacts of the disease on relatives or caregivers

You can address the topics below if they are relevant to you, and add as many aspects or parameters to consider

You can prioritize themes, you can rate their severity using a scale from 1 (less severe) to 5 (most severe)

You can detail the impacts by separating them from "groups", e.g. male/female, child/adult, etc.

– Intellectual or physical fatigue

– Activities of daily living

– Mobility/travel

– Specific problem with genetic diseases affecting other family members

– Work life – Ability to work

– Emotional life

– Sexual life

– Social life

– Psychological impacts

- Financial aspects

– Other aspects

What are the impacts of the disease on loved ones or caregivers?
Maladie: Infection par le VRS chez les nourrissons et hospitalization
Parents/soignants: Qualité de vie, état de santé subjectif, productivité au travail, fonctionnement de la famille, impact psychologique, impact émotionnel, connaissances des parents en matière de santé, structures de soutien, défis/obstacles supplémentaires, sentiment de culpabilité, sentiment d'insécurité

Disease: RSV infection in infants and hospitalisation
Parents/caregivers: Quality of life, subjective health status, work productivity, family

functioning, psychological impact, emotional impact, parental health literacy, support structures, additional challenges/barriers, feeling of guilt, feeling of insecurity

1.2. Currently available treatments (outside of the drug being tested)

The information requested here is important to the Transparency Commission as it evaluates new products against already available treatment options. Notices for reimbursement include a gradation in the therapeutic progress of a new drug with regard to currently available treatments or usual management. This is called "Enhancement of the Rendered Medical Service" (ASMR). The ASMR has five levels ranging from "absent" (ASMR V) to "major" (ASMR I).

Please describe mainly the treatments that have the same indication as the product being evaluated (same disease, same age, same objective, treatment given at the same stage of progression, e.g. first-line or after failure of another treatment, etc.).

Provide a brief description of these treatments, their advantages and disadvantages, and their impact on the quality of life (beneficial or undesirable effects, ease or difficulty of use) and on the patient's care pathway (hospitalization, travel outside the home, frequency of tests related to treatment follow-up, etc.).

What are the current treatments used in the indication mentioned for this file?
For example, drugs, medical devices, rehabilitation, supportive care, psychological support, etc.
Soins de soutien, traitement symptomatique, pour les prématurés et les nourrissons atteints d'affections préexistantes: Immunisation passive; En Allemagne, ce médicament devrait être disponible à partir de l'automne 2024: Le Comité permanent des vaccinations (STIKO) recommande le nirsevimab (Beyfortus) pour tous les nourrissons au cours de la première année de vie, mais le financement n'est pas encore finalisé.

Supportive care, symptomatic treatment, for preterm and infants with preexisting conditions: Passive immunization; In Germany expected from autumn 2024: The Standing Committee on Immunisation (STIKO) recommends Nirsevimab (Beyfortus) for all infants in the first year of life, however, financing not yet finalized.

What are the benefits of currently available treatments?
Avantages: Moins d'infections pendant la saison du VRS pour les enfants nés avant terme et les enfants souffrant de maladies préexistantes.

Benefits: Less infections during RSV season for preterm born children and children with pre-existing conditions.

What are the disadvantages of currently available treatments?
Injections mensuelles pendant la saison du VRS, non disponibles pour tous les nourrissons.

Monthly injections during RSV seasons, not available for all infants.

What are some of the arguments that would allow you to say that the treatments currently offered in this indication do not cover all your needs?
Les traitements actuels ne couvrent pas tous les nourrissons du groupe cible/risque respectif. Le VRS peut constituer une menace pour tous les nourrissons, indépendamment de toute condition médicale préexistante ou coexistante. D'après les chiffres réels, 79 % des enfants hospitalisés pour le VRS étaient auparavant en bonne santé et nés à terme. Dans notre étude ResQ Family, 37% des nourrissons hospitalisés pour VRS en France étaient nés à terme. Dans le monde, 33,8 millions d'épisodes d'infections aiguës des voies respiratoires inférieures induites par le VRS (IALR-VRS) et les IALR-VRS sont une cause majeure de morbidité et de mortalité chez les enfants de moins de 5 ans.

Current treatments do not cover all infants in the respective target/risk group. RSV can be a threat for all infants regardless of any pre/co-existing medical condition. Based on actual numbers, 79% of children hospitalised for RSV were previously healthy and term-born. In our ResQ Family study, 37% infants hospitalized for RSV in France were term-born. Globally, 33.8 million episodes of RSV induced acute lower respiratory infections (RSV-ALRI) and RSV-ALRI is a main cause of morbidity and mortality in children < 5 years**.**

1.3. The drug being evaluated

The categories below are for informational purposes only and may be used in some questions. It is not necessary to complete all of them. The important thing is to let us know about the 3 main improvements or drawbacks that may relate to either of these categories, for example:

- the person's state of health, recovery, lifespan if serious illness;

– quality of life (including impact on intellectual or physical fatigue, activities of daily living, mobility and travel, professional life or work abilities, emotional life, sexual life, social life or other aspects to be specified. In the context of pediatrics, this could include growth, education, fun and sports activities;

- the quality of life of his relatives;

- the use of this treatment;

– the patient's health and life journey;

– others: feel free to add anything else you want.

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_____

1.3.1. Your comments on the indication requested by laboratory

Does the label of the requested indication (which refers to the patients concerned) by the laboratory seem appropriate to you? Is the entire patient population included in this language? Should other patient groups be included? Please justify your response.

Améliorations: État de santé du nourrisson, qualité de vie du patient (nourrisson), qualité de vie des parents/soignants, lutte contre l'utilisation excessive des services de santé pendant la saison du VRS en raison de la pénurie de personnel.

Improvements: Infant's state of health, quality of life of patient (infant), quality of life of parent/caregiver, counteracting excessive utilization of healthcare services during RSV season with staff shortage.

If your opinion is based on the experience of people who have used this treatment, fill in the section "If you have experience..." below (§ 1.3.2).

If you do not know any patients who have used this treatment, only complete the section "If you do not have experience..." (§ 1.3.3).

You can leave either of these topics blank.

1.3.2. If you have experience with the study drug (otherwise go to the next section)

1.3.2.1. Benefits seen with the use of the study drug compared to other treatments currently used:

What are the main improvements seen compared to current treatments?

Examples of improvement (this list gives only a few possible examples): fewer side effects, more efficacy on an aspect of the disease, more comfortable frequency or mode of administration, etc..

Principaux avantages: recommandé pour tous les nourrissons, une seule injection est nécessaire avant le début de la saison contrairement à 5 (mensuellement, pendant la saison) et uniquement disponible pour un groupe spécifique de nourrissons/enfants. Une seule injection signifie également que les nourrissons sont moins gênés par la douleur (injection) et que les parents ont moins d'efforts à fournir et moins de soucis à se faire en termes de visites multiples chez le médecin au cours de la saison.

Main benefits: recommended for all infants, only one injection needed before season starts as compared to 5 (monthly, during season) and only for a specific group of infants/children. One injection also means pain that the infants are less bothered with pain (injection) and less effort and less hassle for parents in terms of multiple doctor's visits during the season.

1.3.2.2. Observed disadvantages of the drug being studied compared to other drugs currently used

What are the main drawbacks of the drug being studied, particularly compared to those of current treatments?
Pour l'Allemagne: Comment adapter les calendriers de vaccination existants pour les nourrissons (nés à terme et en bonne santé) et le financement (en cours d'évaluation).

For Germany: How to adapt existing vaccination schedules for (healthy term born) infants and financing (currently under evaluation).

1.3.3. If you do not have experience with the medication being tested: what are your expectations and concerns?

1.3.3.1. Your expectations for the drug being studied in this assessment

Examples of expectations (this list gives only a few possible examples): fewer side effects, more efficacy on an aspect of the disease, more comfortable frequency or mode of administration, etc.

What are the main improvements expected compared to current treatments ?
Réduction de la gravité des symptômes dus à une infection par le VRS, amélioration de la qualité de vie des parents/soignants, moindre recours aux services de santé.

Reduced severity of symptoms due to an RSV infection, improved quality of life of parents/caregivers, less utilisation of healthcare services.

1.3.3.2. Vos craintes concernant le médicament étudié dans cette évaluation

What are the main fears about this drug, especially compared to current treatments?
Pour l'Allemagne: L'adaptation des calendriers de vaccination existants et le financement (en cours d'évaluation).

For Germany: The adaption of current existing vaccination schedules and financing (currently under evaluation).

2. Questionnaire – Part B (Early Access Records Only)

This section is for Early Access Authorization requests only

If your contribution relates to a notice for reimbursement, please proceed directly to Part C "Summary".

The decision whether or not to allow early access made by the HAS is based on four regulatory criteria:

1. *the rare, serious or disabling nature of the disease treated by the drug for which the early authorization request is made.*
2. *Lack of appropriate treatment available.*
3. *the presumed innovative nature of the medicinal product, in particular with regard to a possible clinically relevant comparator.*
4. *the inability to delay treatment.*

Therefore, in this section, we invite you to position yourself on these questions.

You can use the "other" fields in a free-form way to express yourself.

2.1. Your Position on Early Access Authorization Criteria

What are some arguments that would allow you to say that the disease for which this drug is being evaluated is disabling and/or serious?

Response box (you can enlarge)

What are some arguments that would allow you to say that the treatments currently offered in this indication are not appropriate?

Response box (you can enlarge)

What are the main arguments that allow you to say that the drug being studied seems innovative to you compared to current treatments

This question concerns the innovative nature of the use of the product for which the request for early access authorization is requested.

The innovative nature of the product in the requested indication is based in particular on two points:

– substantial change in efficacy including quality of life, tolerability, convenience or convenience of use or care pathway;

– coverage of an unmet or under-covered medical need (e.g. : Suitable for use in children).

Response box (you can enlarge)

2.2. Your position on the data to be collected by patients in case early access to the drug is granted

It is essential during early access to carefully observe the use and effects of the drug to better understand it and evaluate its efficacy and adverse effects in "real life".

Data on drug use and effects are collected from patients in two ways:

- by the doctor during the consultations: the doctor prescribing this drug will ask patients questions about the condition in which they feel with the treatment;

– by patients themselves between visits: patients (and/or their relatives in some cases) will receive one or more questionnaires online or in paper format in order to collect health data themselves and more particularly quality of life. These quality of life questionnaires must be completed by the patients themselves, without interpretation by the physician or third parties.

How this monitoring and data collection is organized is described in detail in a specific document called "Protocol for Therapeutic Use – Data Collection", or: PUT-RD.

We invite you to give us your opinion on what data or information would be relevant to collect from the perspective of patients and/or caregivers.

In this section, we ask you to express the types of data and how to collect it that are most relevant to you. It is not useful to mention purely medical data such as biological parameters.

2.2.1. The data and information that is important to collect

What is the key information patients might collect themselves to help better understand (qualitatively and/or quantitatively) the efficacy and safety of the treatment being evaluated?
Response box (you can enlarge)

What are the conditions for patients to best collect the requested information (collection at home, with the help of a caregiver in the hospital, collection by relatives, collection with the help of an expert patient, combination of several collection methods, etc. ?)
Response box (you can enlarge)

2.2.2. Your Opinion on Therapeutic Use Protocol - Data Collection

The submission of a "therapeutic use and data collection protocol" (PUT-RD) is a legal obligation for the pharmaceutical company submitting an early access request. These usage data will increase knowledge of the drug in routine clinical practice.

The purpose of the PUT-RD is to collect data and monitor patients; it must mention what will be collected for this purpose.

The PUT-RD is not published on our site. The HAS may decide to transmit it as it was drafted by the manufacturer during its filing of files to patient associations incorporated in a legal entity.

If you would like to review this document for comment in this section, please send us a request email at contact.contribution@has-sante.fr. The consultation of the document is subject to the signing of a confidentiality commitment, and will only be approved for associations incorporated in a legal entity. The document is usually sent within 72 hours.

Did you consult the draft protocol for therapeutic use – Data collection?
☐ Yes

☐ No

Do you have any comments or supplements related to the protocol for therapeutic use – Data collection?

Response box (you can enlarge)

3. Questionnaire – Part C : Hearing Request

The Transparency Commission may decide to interview one or more user associations or groups when reviewing the application.

Here you can express interest in such a hearing (in addition to this written contribution).

CAUTION: Hearings before the Transparency Committee are usually granted under early access authorizations; they are exceptional under reimbursement.

Would you like to be heard?

☒ Yes*

☐ No

*Communication en anglais de préférence

*Communication in English preferred

Why?

Le groupe cible des patients de l'EFCNI (prématurés et enfants mal nés) est l'un des principaux groupes à risque et nous sommes pleinement conscients de la maladie ainsi qu'en contact avec les professionnels de la santé, les chercheurs et le grand public pour les sensibiliser. Nous savons également, d'après l'expérience des parents, à quel point une infection par le VRS, avec ou sans hospitalisation de l'enfant, peut avoir un impact sur la qualité de vie des parents et des personnes qui s'occupent de l'enfant. De plus amples informations à ce sujet peuvent être obtenues ici, y compris les résultats de l'étude ResQ Family en France

EFCNI_Positionpaper_RSV_EN_web.pdf, RSV: Are you RSV aware? – EFCNI, EFCNI_ResQFamily_Mini_Report_FR.pdf

The patient target group of EFCNI (pre-term and ill- born infants) is one of the major risk groups and we are fully aware of the disease as well as in contact exchange with healthcare professionals, researchers and the main public to raise awareness. We also know from parents' experiences how badly an RSV infection with or without hospitalisation of the child can impact the quality of life of parents/caregiver. Further information in this regard can be obtained from here, including the results of the ResQ Family study in France

EFCNI_Positionpaper_RSV_EN_web.pdf, RSV: Are you RSV aware? – EFCNI, EFCNI_ResQFamily_Mini_Report_FR.pdf

4. Questionnaire – Part D : Summary

List the most important points of your contribution. For example:

– The biggest challenges of living with the disease are...

– Currently available therapies are (in) adequate because ...

– The study drug meets (little) patients' needs and expectations because ...

– The most important unmet treatment needs are ...

This list is of course not exhaustive.

Le principal avantage du nouveau médicament à l'étude est que tous les enfants seront protégés d'une évolution grave de la maladie, qui a également un impact sur la qualité de vie et le bien-être des parents et des soignants. En outre, le nirsevimab pourrait atténuer la situation tendue dans les cliniques pédiatriques pendant la saison du VRS.

The main benefit of the new study drug is that all children will be protected from a severe disease course which also impacts the quality of life and wellbeing of parents and caregivers. Furthermore, Nirsevimab could ease the tense situation in paediatric clinics during the RSV season.

5. Questionnaire – Part E : Your other remarks

If you would like to complete the information that you consider useful for the Transparency Commission, please use this part freely.

Pour l'Allemagne: Une adaptation judicieuse du calendrier des vaccinations pour la vaccination contre le VRS ainsi que le financement doivent faire l'objet d'une réflexion approfondie avant d'être mise en œuvre.

For Germany: A sensible adaptation of the vaccination schedule for RSV immunisation as well as the financing must be thoroughly thought through before implementation.

6. Questionnaire – Part F : Methods

Methods used to complete this questionnaire

Indicate the method used to inform the different parts of this questionnaire (e.g. survey, social networks, working group, testimonials, qualitative interviews of patients or relatives who have had access to treatments during clinical trials, telephone calls, number of participants, international exchanges with associations of countries where the treatment is already marketed, with the periods concerned).

In which methods have you completed the disease impact chapters and currently available treatments?
Sans objet car le questionnaire ne portait pas sur le traitement.

Not applicable as the questionnaire was not about the treatment.

How did you collect patient experience with this treatment?
Sans objet car le questionnaire ne portait pas sur le traitement.

Not applicable as the questionnaire was not about the treatment.

Who contributed significantly to writing the contribution?#

Dr. Christina Tischer

Has the association received outside support to support its contribution? If so, which ones?

Aucun.

None.

Can you give us an estimate of the time it took to write this contribution?
40 minutes.

Did you have any difficulties completing this questionnaire, and if so, which ones?
Aucun.

None.

Acknowledgement

Thank you very much for your input and time. We know these are important. Your contribution will be considered by the Transparency Commission. It will be distributed to all members of the latter in the same way as the other documents in the file, and will be the subject of an oral presentation by members of committees appointed as members of a user association before the deliberations.

Questionnaire Design

This questionnaire was built in collaboration with association representatives through a dedicated working group. For more information, Click here