Benefits of NPPs treatment for allergic respiratory diseases: *the French ERAPP prospective cohort study*

Evaluation of patients' expectation and benefits of sublingual Named-Patient Products in the treatment of allergic respiratory diseases: the French ERAPP prospective cohort study

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ABSTRACT

Background

In France, **Named-Patient Products (NPPs)** are used for the treatment (**Allergen Immunotherapy**, or **AIT**) of allergic respiratory diseases. Every year, **300,000** patients are treated with NPPs, with 100,000 new patients per year. The French Health Technology Assessment agency requested data to evaluate the effectiveness of sublingual NPPs.

OBJECTIVE

The objective of the study was to evaluate the impact of sublingual NPPs on patient-reported outcomes.

METHODS

- French prospective cohort study of patients with a prescription of NPPs between September 2020 and February 2022 and followed-up at 6 and 12 months.
- **Index date:** date of response of the 1st questionnaire
- Patients (adults / adolescents ≥ 12 years old, and children with parent's help) answered an electronic medical questionnaire adapted to the patient's age including the **rating of personal importance of 25 treatment needs** on a five-point Likert scale from 0 (not important) to 4 (very important) (Figure 2)
- **Patients initiating an AIT** received **PNQ** (Patient Needs Questionnaire) at index date and **PBQ** (Patient Benefit Questionnaire), extent to which these needs were met by treatment, at 6 and 12 months.
- The primary endpoint was the **Patient Benefit Index** (**PBI**), ranging from 0 to 4, which provides a weighted overall benefit parameter (of the PNQ and PBQ).

$$PBI\ at\ month\ i = \sum [(IQPI_n / \sum IQPI_n)\ X\ IQPim_n]$$

with :

$IQPI_n$ = *item n of questionnaire at inclusion*

$IQPim_n$ = *item n of questionnaire at month i*

i = 6, 12 month, and n = (1,… 25)

A PBI ≥ 1 is considered to reflect a treatment benefit

- Patients answered other validated and standardised questionnaires: allergic rhinitis, severity and control of rhinitis (ARIA, T5SS and rhinitis symptoms questionnaire), severity and control of asthma (ACT, GINA), sleep (Epworth), quality of life (5-point Likert score), therapeutic compliance (GIRERD)

RESULTS

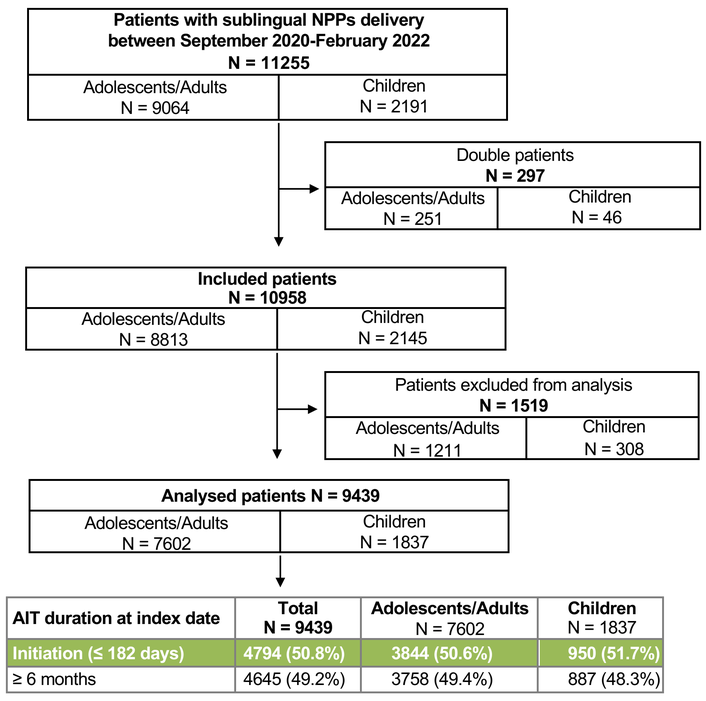
- **4,794 patients** starting sublingual NPPs were included: 3,844 adults / adolescents ≥12 years old (39.5% men), 950 children (65.8% boys), with a mean age of 34.2 years and 8.4 years, respectively.
- At inclusion, most patients had an indication of NPPs for **allergic rhinitis** (90.2% adolescents-adults and 82.7% children), 19.7% and 27.2% had asthma, 55.7% of adults had a duration of allergy>10 years.
- 1,190 adolescents-adults and 387 children had complete data at 6 and 12 months.
- **A treatment benefit was observed at 6 and 12 months:** PBI was ≥1 in 81.3% of adults-adolescents and 78.0% of children at 6 months, 84.1% and 83.2% at 12 months.
- Among patients who started sublingual NPPs and were evaluable at initiation and 6 or 12 months, 45.1% and 54.0% of adults-adolescents, respectively, and 47.4% and 56.9% of children, respectively, reported that their **quality of life had often or always improved** since taking NPPs.
- Results regarding other secondary outcomes suggested no major trends at 6 and 12 months.

CONCLUSION

This study highlights benefits of NPPs treatment for allergic respiratory diseases. Further analyses are planned to evaluate the effect of NPPs on trends in healthcare consumption using this cohort matched to the French healthcare insurance system database (SNDS).

Conflict of interest statement

This study was funded by an unrestricted grant from SFA (Société Française d'Allergologie). P Demoly reports having received indirect fees from ALK, AstraZeneca, Chiesi, GlaxoSmithKline, Menarini, Puressentiel, Stallergenes Greer, ThermoFisherScientific, Viatris and Zambon. D Caimmi reports having received fees or honorarias from ALK, AstraZeneca, GlaxoSmithKline, Puressentiel, Sanofi Genzyme, Stallergenes Greer, and Viatris.
For all other Authors, Bordeaux PharmacoEpi is an independent research platform of the Bordeaux University and its subsidiary the ADERA, which performs financially supported studies for public and private partners, in compliance with the ENCePP Code of Conduct.



AIT duration at index date	Total N = 9439	Adolescents/Adults N = 7602	Children N = 1837
Initiation (≤ 182 days)	4794 (50.8%)	3844 (50.6%)	950 (51.7%)
≥ 6 months	4645 (49.2%)	3758 (49.4%)	887 (48.3%)

NPPs: Named-Patient Products AIT: Allergen ImmunoTherapy

Figure 1. Selection of patients for data analysis

For each of the following, please tick the box that best describes the help you expect (**PNQ**) / have received (**PBQ**) from your NPP treatment:

	Not concerned	Not important	Not very important	Fairly important	Important	Very important
To be able to stay outdoors without symptoms	☐	☐	☐	☐	☐	☐
To no longer have a runny or stuffed-up nose	☐	☐	☐	☐	☐	☐
To not have itching on the eyes, nose or palate anymore	☐	☐	☐	☐	☐	☐
To note have burning or watery eyes anymore	☐	☐	☐	☐	☐	☐
To be healed of all symptoms	☐	☐	☐	☐	☐	☐
To be able to breathe through my nose nore freely	☐	☐	☐	☐	☐	☐
To feel less fatigued or groggy	☐	☐	☐	☐	☐	☐
To have confidence in the therapy	☐	☐	☐	☐	☐	☐
To have an easily applicable treatment	☐	☐	☐	☐	☐	☐
To be able to sleep better	☐	☐	☐	☐	☐	☐
To experience a greater enjoyment of life	☐	☐	☐	☐	☐	☐
To be able to engage in normal leisure activities	☐	☐	☐	☐	☐	☐
To feel less depressed	☐	☐	☐	☐	☐	☐
To be able to concentrate better at work	☐	☐	☐	☐	☐	☐
To not have sneezing impulses	☐	☐	☐	☐	☐	☐
To have no fear that the disease will become worse	☐	☐	☐	☐	☐	☐
To be more productive in everyday life	☐	☐	☐	☐	☐	☐
To be less dependent on doctor and clinic visits	☐	☐	☐	☐	☐	☐
To have fewer side effects	☐	☐	☐	☐	☐	☐
To feel less irritated	☐	☐	☐	☐	☐	☐
To have fewer out-of-pocket treatment expenses	☐	☐	☐	☐	☐	☐
To need less time for daily treatment	☐	☐	☐	☐	☐	☐
To feel more comfortable showing yourself	☐	☐	☐	☐	☐	☐
To be less burdened in your partnership	☐	☐	☐	☐	☐	☐
To be able to have a normal sex life*	☐	☐	☐	☐	☐	☐

** tick "not concerned" if you are not concerned; PNQ: Patient Needs Questionnaire; PBQ: Patient Benefit Questionnaire*

Figure 2. *:* Patient Needs/Benefit Questionnaire (PNQ/PBQ)

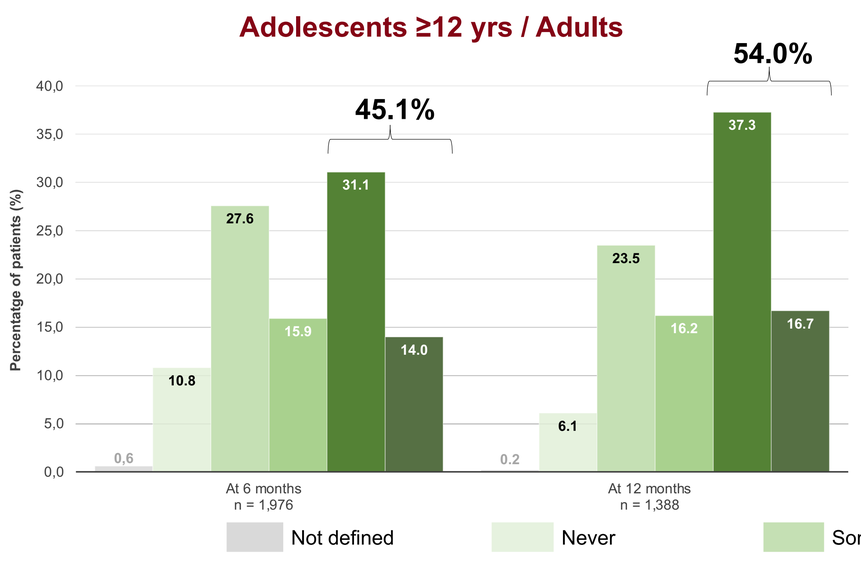
Adolescents ≥12 yrs / Adults		Children <12 yrs
N = 3844		N = 950
39.5%	**Male (%)**	65.8%
34.2 ± 14.6 yrs	**Mean age ± SD (in years)**	8.4 ± 1.9 yrs
62.7%	**Residence in urban area (%)**	49.8%
76.9%	**Non smoker (%)**	98.4%
90.2%	**NPPs indication: allergic rhinitis (%)**	82.7%
55.7%	**Allergy history >10 yrs (%)**	0.8%
19.7%	**Asthma at inclusion (%)**	27.2%

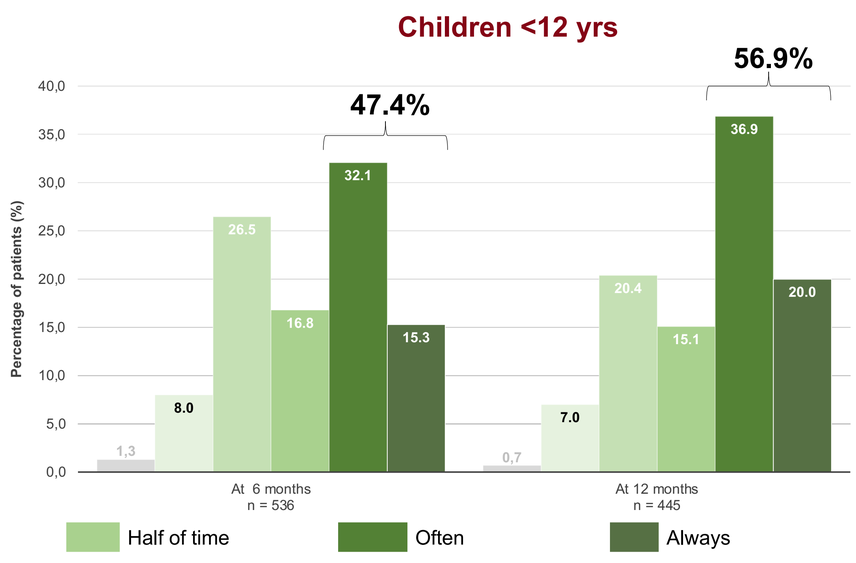
Positive Patient Benefit Index (PBI) during follow-up

The PBI was calculated in patients with available information in the 3 questionnaires: inclusion, 6 and 12 months

Adolescents ≥12 yrs / Adults		Children <12 yrs
N = 1190	*At 6 months*	N = 386
81.3%	**PBI ≥ 1 (%)**	**78.0%**
2.0 ± 1.0	Mean PBI ± SD	1.9 ± 1.0
2.0 [1.2;2.7]	Median PBI [CI 95%]	1.8 [1.1;2.6]
	At 12 months	
84.1%	**PBI ≥ 1 (%)**	**83.2%**
2.1 ± 1,0	Mean PBI ± SD	1.9 ± 0.9
2.1 [1.3;2.8]	Median PBI [CI 95%]	1.8 [1.2;2.6]

Improvement in quality of life since taking NPPs treatment





SD Standard Deviation, CI Confidence Interval

Colloque des Données de Santé en Vie Réelle, Rive Montparnasse - Paris, 20 juin 2024

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Évaluation des attentes et des bénéfices chez les patients traités par APSI pour des maladies respiratoires allergiques : l'étude de cohorte prospective française ERAPP

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Déclaration d'intérêt: Cette étude a été financée par une subvention sans restriction de la SFA (Société Française d'Allergologie).

P Demoly déclare avoir reçu des honoraires indirects de ALK, AstraZeneca, Chiesi, GlaxoSmithKline, Menarini, Puressentiel, Stallergenes Greer, ThermoFisherScientific, Viatris et Zambon.

D Caimmi déclare avoir reçu des honoraires de ALK, AstraZeneca, GlaxoSmithKline, Puressentiel, Sanofi Genzyme, Stallergenes Greer et Viatris.

Pour tous les autres auteurs, Bordeaux PharmacoEpi est une plateforme de recherche indépendante de l'Université de Bordeaux et de sa filiale l'ADERA, qui réalise des études soutenues financièrement pour des partenaires publics et privés, en conformité avec le Code de Conduite de l'ENCePP.

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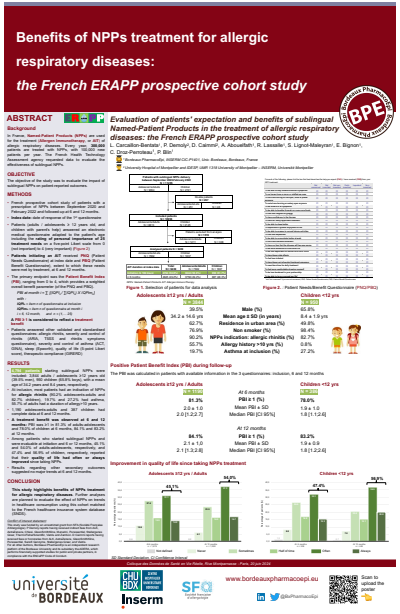

- **APSI** Allergènes Préparés Spécialement pour un seul Individu
  - Traitements d'immunothérapie allergénique (ITA) (allergies respiratoires, asthme, rhinites)
  - Non soumis à l'AMM *(enregistrement ANSM, article L. 4211-6 du CSP)*
  - France : 300 000 patients traités par APSI (<5% des patients allergiques)
  - Juin 2018 : déremboursement des produits sous-cutanés pour les allergènes respiratoires, et remboursement à 30% des formes sous-linguales (15 % pour les comprimés)

HAS HAUTE AUTORITÉ DE SANTÉ : Réévaluation prévue en 2013, demande de preuves de l'intérêt de garder ces produits remboursés

→ SFA Société française d'allergologie : Porteur de 3 projets dont l'étude ERAPP

ERAPP : Étude de cohorte prospective française de patients ayant reçu une prescription d'APSI entre sept 2020 et fév 2022 avec un suivi à 6 et 12 mois

- Objectif principal : évaluer l'évolution du ressenti des patients adolescents ou adultes et des enfants (<12 ans)
- Méthode : **PBI** Patient Benefit Index calculé à partir
  - du **PNQ** (besoins du patient à l'inclusion)
  - du **PBQ** (mesure de la satisfaction de ces besoins à 6 et 12 mois)

  → **Bénéfice apporté par le traitement APSI si PBI ≥ 1**
- **9 439** patients inclus analysés, dont **4 794 inclus** **à l'initiation du traitement APSI**
- **Dès 6 mois de traitement, Bénéfice ressenti (PBI ≥ 1)** pour **81,3 %** des ado/adultes et **78,0 %** des enfants **initiant un traitement APSI** (respectivement 84,1 % et 83,2 % à 12 mois)
- **Amélioration de la qualité de vie des patients**

INFO *A suivre… Chainage des données ERAPP avec les données du SNDS pour évaluer l'effet des APSI sur l'évolution de la consommation de soins*