A registry-based non-interventional post-authorization safety study to evaluate the long-term safety of dupilumab in children aged ≥6 months to <6 years with moderate-to-severe atopic dermatitis using the PEDISTAD registry: a cohort design

First published: 09/05/2024

Last updated: 10/03/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS1000000149

Study ID

1000000149

DARWIN EU® study

No

Study countries

- [ ] Argentina
- [ ] Australia
- [ ] Belgium
- [ ] Brazil
- [ ] Canada
- [ ] China
- [ ] Colombia
- [ ] Denmark
- [ ] France
- [ ] Greece
- [ ] Israel
- [ ] Italy
- [ ] Japan
- [ ] Korea, Republic of
- [ ] Mexico
- [ ] Netherlands
- [ ] Norway
- [ ] Portugal
- [ ] Russian Federation
- [ ] Spain
- [ ] United States

Study description

This registry-based PASS aims to evaluate the long-term safety of dupilumab in children who initiate dupilumab at age ≥6 months to <6years old, using data from the PEDISTAD disease registry of pediatric patients with moderate-to-severe atopic dermatitis (AD).

It is expected that most pediatric patients will have prior exposure to other therapies for AD before initiation of dupilumab or may have overlapping usage with other AD therapies at dupilumab initiation.

For this reason, the cohort of dupilumab initiators will be referred to as the DUPI-All cohort, to communicate that the safety profile of dupilumab initiators may be confounded by prior therapies.
A sub-cohort of dupilumab initiators, restricted to those who only have prior use of systemic corticosteroids or high potency topical corticosteroids will be referred to as the DUPI-Steroid cohort.
The rationale is to have a sub-cohort of dupilumab initiators that is less confounded by use of prior non-steroid immunosuppressant therapies. A sub-cohort of dupilumab initiators that is naïve to all other AD therapies listed in this protocol will also be created, referred to as the Pure-DUPI cohort.
This sub-cohort will reflect the least confounded cohort. The numbers included will depend on real-world drug utilization patterns. To provide context for the long-term safety of dupilumab, safety data will also be described for a cohort of children enrolled in the PEDISTAD disease registry who initiate systemic corticosteroids, UV therapy, immunosuppressants (cyclosporine, methotrexate, mycophenolate and azathioprine), JAK inhibitors (abrocitinib, upadacitinib, tofacitinib, baricitinib), other systemic biologic treatments for moderate-to-severe AD (e.g., tralokinumab) or high potency TCS at age ≥6 months to <6 years old (referred to throughout as the Other AD Therapies cohort).

Study status

Ongoing

Research institutions and networks

Institutions

Sanofi Winthrop Industrie

IQVIA

United Kingdom

First published: 12/11/2021

Last updated: 22/04/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Sydney Willis

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 15/12/2022

Actual: 15/12/2022

Study start date

Planned: 30/09/2024

Actual: 25/04/2024

Data analysis start date
Planned: 30/09/2024

Actual: 20/08/2024

Date of final study report
Planned: 30/09/2032

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Sanofi Winthrop Industrie

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Study design:

This registry-based PASS is an international, observational, registry-based cohort study, using data collected in the ongoing PEDISTAD registry, which enrolls and follows patients aged <12 years old with moderate-to-severe AD.

Main study objective:

Primary objective: To describe the long-term safety of dupilumab in terms of the incidence rate of safety outcomes (adverse events [AEs] and serious adverse events [SAEs]) among patients in the DUPI-All cohort and separately, if sufficient sample size, in the DUPI-Steroid and Pure DUPI sub-cohort.

Secondary Objectives include:
a) To describe the patient characteristics, severity of AD by clinician assessment and by PROs, medical history and selected comorbidities at index date for patients in the DUPI-All cohort and separately, if sufficient sample size, in the DUPI-Steroid and Pure-DUPI sub-cohorts;
b) to describe the patient characteristics, severity of AD by clinician assessment and by PROs, medical history and selected comorbidities at index date for the

patients in the Other AD therapies cohort.
c) to describe the AD drug utilization up to and after the index date (i.e., initiation date of cohort-defining treatment) for patients in the DUPI-All cohort and the Other AD therapies cohort;
d) to describe the incidence rate of safety outcomes (AEs and SAEs) among patients in the Other AD therapies cohort.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

DUPIXENT

Study drug International non-proprietary name (INN) or common name

DUPILUMAB

Anatomical Therapeutic Chemical (ATC) code

(D11AH05) dupilumab

dupilumab

Medical condition to be studied

Dermatitis atopic

Population studied

Short description of the study population

Patients aged >=6 months to <6 years with moderate-to-severe atopic dermatitis

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Age groups

Paediatric Population (< 18 years)

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Estimated number of subjects

515

Study design details

Setting

Secondary use of data collected in the global Pedistad AD disease registry. All patients are treated according to routine clinical care, and all follow-up conducted as per routine care.

The PEDISTAD registry was initiated in September 2018 with an intended prospective follow-up of five years. In 2023, follow-up was extended to 10 years from enrollment. Patients are aged <12 years old with moderate-to-severe AD whose disease is not adequately controlled with topical therapies or for whom those therapies are not medically advisable. During the follow-up period, data are collected at visits that take place as per routine clinical care, planned to take place twice per year, approximately six months apart, which is consistent with typical standard of care in this patient population. Patients are included in the DUPI PEDISTAD-registry-based PASS on the date of initiating a cohort-defining medications (the index date).

Comparators

DUPI-ALL cohort: Initiated treatment with dupilumab, with index date at or after PEDISTAD enrollment date; no restriction based on usage of prior or overlapping “other AD therapies”; aged ≥6 months to <6 years at index date.
DUPI-Steroid sub-cohort: sub-cohort of the DUPI-ALL cohort; initiated treatment (first ever) with dupilumab, with index date at or after PEDISTAD enrollment date; prior or overlapping use of SCS or high potency TCS at index date - prior or overlapping use of other systemic agents at the index date is not permitted; aged ≥6 months to <6 years at index date. Pure-DUPI sub-cohort: subcohort of the DUPI-ALL cohort; initiated treatment (first ever) with dupilumab with index date at or after PEDISTAD enrollment date; no prior or overlapping use of any "other AD therapy" at index date; aged ≥6 months to <6 years at index date.
Other AD therapies cohort: Initiated treatment with SCS, UV therapy, immunosuppressants, JAK inhibitors , other systemic biologic treatments for moderate-to-severe AD (e.g., tralokinumab) or high potency TCS with index date at or after PEDISTAD enrollment date; aged ≥6 months to <6 years at index date. Prevalent Dupilumab Users cohort: receiving ongoing dupilumab treatment at enrollment into the PEDISTAD registry (prevalent users), aged ≥6 months to <6 years at index date.

Outcomes

Safety outcomes (AEs and SAEs), exposure treatment details, patient characteristics, AD disease characteristics, medical history, comorbidities.

Data analysis plan

The primary analysis will include calculation of incidence rates with 95% confidence intervals (CI) presented for AEs and SAEs, overall and categorized by intensity and according to the Medical Dictionary for Regulatory Activities (MedDRA) groupings (e.g., by system organ class (SOC) and/or preferred term (PT)). The incidence rates will be provided for the DUPI-All cohort, and

separately if sufficient sample size, the DUPI-Steroid and Pure-DUPI cohorts. The incidence rate will be calculated as the number of events divided by number of exposed person-years and will be expressed as incidence rate per 100 person-years. All events with onset at or after index date will be included and persons-years will be calculated as the time-period (in years) between index date and the end of exposure for each patient.
For the secondary analyses, the patient characteristics, severity of AD by clinician assessment and patient/caregiver assessment, prior and concomitant diseases will be described among the DUPI-All and Other AD therapies cohorts using descriptive statistics. The AD drug utilization patterns will be described using descriptive statistics as well. Confidence intervals, whenever deemed appropriate, will be 2-sided with a confidence probability of 95%, unless otherwise specified.

Furthermore, the incidence rates of AEs and SAEs with 95% CI, overall and categorized according to MedDRA (for example by SOC and/or PT) and by intensity, will be presented for the Other AD therapies cohort.

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data source(s), other

Pedistad AD Disease Registry

Data sources (types)

Disease registry

Non-interventional study

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Yes

Check completeness

Yes

Check stability

Unknown

Check logical consistency

Yes

Data characterisation

Data characterisation conducted

Unknown