Pediatric Clinical Trial Program in Progress: A Phase 3 Study and Long-term Extension Study to Evaluate the Efficacy and Safety of Ritlecitinib in Children 6 to <12 years of Age With Severe Alopecia Areata

Bianca Maria Piraccini,[1] Jennifer Soung,[2,3] Thierry Passeron,[4,5] Wenyu Wu,[6] Misaki Kinoshita-Ise,[7] Mercedes E. Gonzalez,[8] G. S. H. Ramakanth,[9] Amita Priya Dommalapaty,[9] Dalia Wajsbrot,[10] Helen Tran,[11] Monica Lindsay,[10] Robert Wolk[10]

[1]Private Dermatology Practice, Bologna, Italy; [2]Southern California Dermatology, Inc, Santa Ana, CA, USA; [3]Harbor University of California Los Angeles, Torrance, CA, USA; [4]Department of Dermatology, CHU Nice, Université Côte d'Azur, Nice, France; [5]C3M, INSERM U1065, Université Côte d'Azur, Nice, France; [6]Department of Dermatology, Huashan Hospital, Fudan University, Shanghai, China; [7]Department of Dermatology, Kyorin University Faculty of Medicine, Tokyo, Japan; [8]Pediatric Skin Research, Miami, FL, USA; [9]Pfizer Healthcare India Private Limited, Chennai, India; [10]Pfizer Inc, Groton, CT, USA; [11]Pfizer Inc, New York, NY, USA

BACKGROUND

- Alopecia areata (AA) is an autoimmune disease characterized by patchy or complete nonscarring hair loss on the scalp, with or without additional loss of facial and/or body hair[1,2]
  - AA is associated with considerable impacts on health-related quality of life. Approximately 31% to 48% of patients present with AA before the age of 20 years[1,2]
- Ritlecitinib, an oral, selective dual inhibitor of JAK3 and the TEC family kinases, is approved for treatment of severe AA in adults and adolescents 12 years and older in the US, EU, Japan, China, and several other countries.[3] There are no treatments currently approved for pediatric patients aged <12 years with AA
  - The approved dose of ritlecitinib in both adults and adolescents is 50 mg once daily (QD)
- A phase 1 study (NCT05650333) characterized the pharmacokinetics of ritlecitinib 20 mg in pediatric patients aged 6 to <12 years with AA[4] and informed the selection of doses for evaluation in phase 3
- The efficacy and safety of ritlecitinib in children aged 6 to <12 years with severe AA are currently under investigation in an ongoing phase 3 study (NCT07029711) and long-term extension (LTE) study (NCT07029828)
  - The phase 3 study is a randomized, double-blind, placebo-controlled trial that evaluates the efficacy, safety, and tolerability of ritlecitinib 50 mg and 30 mg compared with placebo for 24 weeks in pediatric patients aged 6 to <12 years with ≥50% scalp hair loss due to AA
  - Pediatric patients who complete the phase 3 study or who have completed the phase 1 study may enroll in the double-blind LTE study, which seeks to further assess the long-term safety and efficacy of ritlecitinib in pediatric patients for up to 36 months

OBJECTIVE

- The primary objective of the phase 3 study is to evaluate the efficacy of ritlecitinib compared with placebo on regrowth of lost scalp hair in pediatric patients with severe AA
  - The secondary objectives are to further evaluate the safety, tolerability, acceptability, and palatability of ritlecitinib and to evaluate the effect of ritlecitinib on patient-centered outcomes
- The primary objective of the LTE study is to evaluate the long-term safety and tolerability of ritlecitinib in pediatric patients with severe AA who have completed the phase 1 or phase 3 studies of ritlecitinib
  - The secondary objectives are to evaluate the long-term efficacy of ritlecitinib and the effect of ritlecitinib on patient-centered outcomes

MATERIALS AND METHODS

Study populations

- Key inclusion and exclusion criteria for the phase 3 study and the LTE study are shown in **Figure 1**
  - For patients in the LTE study, additional exclusion criteria included occurrence of safety-related events during the phase 1 or phase 3 studies or in the period between those studies and LTE study that required permanent discontinuation
- Continuation criteria for the LTE study are shown in **Figure 2**
  - Patients who do not meet these continuation criteria will be discontinued from ritlecitinib treatment

Figure 1. Key inclusion and exclusion criteria

Key inclusion criteria

- **Age 6 to <12 years**
- Diagnosis of AA, including alopecia totalis and alopecia universalis, with **≥50% scalp hair loss due to AA** (Severity of Alopecia Tool [SALT] score of ≥50)
- Current AA **episode duration of ≥12 months**
- **Varicella vaccination** (2 doses) or evidence of prior exposure to varicella-zoster virus

*For study patients in the **EU/UK only**, in the **phase 3 study**:*

- A history of clinical response **failure to AA treatment**

Key exclusion criteria

- Any present or history of malignancies, lymphoproliferative disorders, or immunodeficiency disorders
- Untreated or inadequately treated active or latent *Mycobacterium tuberculosis* infection
- History of severe cytomegalovirus infection, herpes zoster or disseminated herpes simplex, hepatitis B virus, hepatitis C virus, or infection with HIV
- History of any infection requiring hospitalization or parenteral antimicrobial therapy or otherwise judged as clinically significant within the prior 3 months
- Vaccination with live attenuated replication-competent vaccine within 6 weeks of first dose of study intervention

AA, alopecia areata.

Figure 2. Continuation criteria for the LTE study

For patients who received ritlecitinib in the phase 3 study:

- Improvement of ≥50% in SALT score at Month 3 of the LTE study compared with the SALT score on Day 1 of the phase 3 study
- SALT score of ≤20 at Month 6 of the LTE study

For patients who received ritlecitinib in the phase 1 study *or* were assigned to placebo in the phase 3 study:

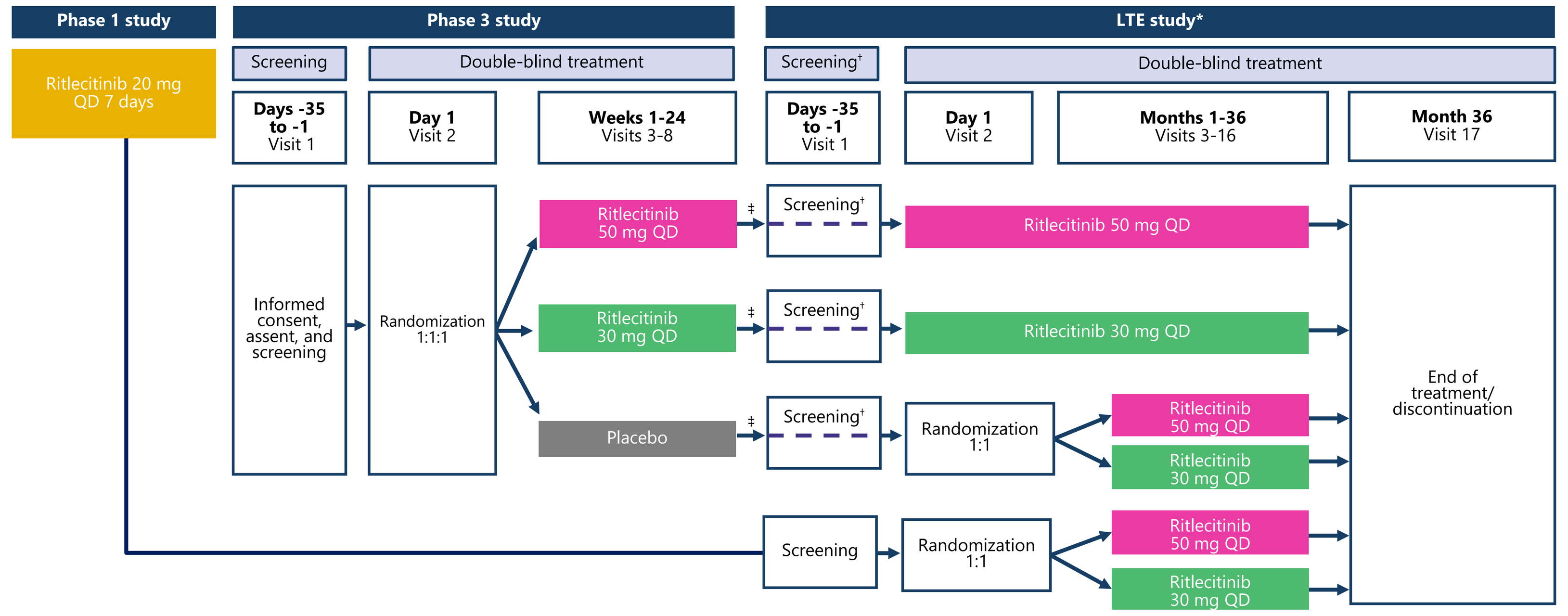
- Improvement of ≥50% in SALT score at Month 9 of the LTE study compared with the SALT score on Day 1 of the LTE study
- SALT score of ≤20 at Month 12 of the LTE study

LTE, long-term extension; SALT, Severity of Alopecia Tool; SALT score of ≤20, ≤20% scalp without hair.

Study designs

- The systemic exposure data generated in adult and adolescent patients[3,5-9] and data from the phase 1 study[4] were used to derive the doses providing equivalent exposures for children 6 to <12 years old
  - The selected doses of ritlecitinib were intended to be equivalent to the 50-mg and 30-mg doses in adults and adolescents
- For the phase 3 study, approximately 225 pediatric patients will be enrolled
  - Patients will be randomized 1:1:1 to ritlecitinib 50 mg QD, ritlecitinib 30 mg QD, or placebo (**Figure 3**)
  - Patients completing the 24-week treatment period may have the option to enter the LTE study
- For the LTE study, it is estimated that ≈140 pediatric patients completing the phase 1 study or phase 3 study will be enrolled
  - Patients who receive ritlecitinib 50 mg QD or 30 mg QD in the phase 3 study will continue receiving the same ritlecitinib dose for up to 36 months (**Figure 3**)
  - Patients who receive placebo in the phase 3 study and all patients from the phase 1 study will be randomized 1:1 to ritlecitinib 50 mg QD or 30 mg QD for up to 36 months

Figure 3. Study designs for the phase 3 study and the LTE study of ritlecitinib in pediatric patients


Phase 1 study
Phase 3 study
LTE study*
Ritlecitinib 20 mg QD 7 days
Screening
Double-blind treatment
Screening†
Double-blind treatment
Days -35 to -1 Visit 1
Day 1 Visit 2
Weeks 1-24 Visits 3-8
Days -35 to -1 Visit 1
Day 1 Visit 2
Months 1-36 Visits 3-16
Month 36 Visit 17
Informed consent, assent, and screening
Randomization 1:1:1
Ritlecitinib 50 mg QD
Ritlecitinib 30 mg QD
Placebo
‡
Screening†
Ritlecitinib 50 mg QD
Ritlecitinib 30 mg QD
Randomization 1:1
Screening
End of treatment/ discontinuation

LTE, long-term extension; QD, once daily.
*Patients who meet the efficacy criteria for discontinuation will receive placebo and remain in the study. †Additional screening visit will not be applicable if the end of treatment/Week 24 visit of the phase 3 study will overlap with screening of the LTE study, but screening procedures, such as signing of informed consent, will still be performed. ‡If the patient is ineligible for the LTE study or has early study discontinuation, they receive 4 weeks of off-treatment follow-up.

Endpoints

- Endpoints for the phase 3 and LTE studies are shown in **Tables 1-3**

Table 1. Key endpoints for the phase 3 study of ritlecitinib in pediatric patients

	US and Countries Following the US Analysis Plan	EU/UK and Countries Following the EU/UK Analysis Plan
Primary endpoint	SALT ≤20 response at Week 24	SALT ≤10 response at Week 24
Key secondary endpoint	Not applicable	PGI-C response* at Week 24

PGI-C, Patient Global Impression of Change; SALT, Severity of Alopecia Tool; SALT ≤10, ≤10% scalp without hair; SALT ≤20, ≤20% scalp without hair.
*PGI-C response defined as "moderately improved" or "greatly improved."

Table 2. Secondary endpoints for the phase 3 study of ritlecitinib in pediatric patients

Secondary Endpoints for All Countries	
Efficacy endpoints	CFB in SALT score*
	SALT ≤10 response*,†
	SALT ≤20 response*,†
	ELA response*,‡
	EBA response*,§
Patient-centered outcome endpoints	PGI-C response‖
	Improvement from baseline for each AAPPO item response (11 endpoints)*
	CFB in AAPPO activity limitation and emotional symptoms scores*
	CFB in PROMIS Parent Proxy Depressive Symptoms T-score*
	CFB in PROMIS Parent Proxy Anxiety Symptoms T-score*
	CFB in modified CDLQI total score*
Neuropsychological status	CFB† in BRIEF®2 T-scores for 3 index scores (BRI, ERI, CRI)*
Safety endpoints	Incidence of treatment-emergent AEs (including audiological and neurological)
	Incidence of SAEs and AEs leading to permanent discontinuation from the study
Taste assessment	Acceptability and palatability assessment¶
Pharmacokinetic characterization	Plasma concentration of ritlecitinib at 1 h (±15 min) and 3 h (±30 min) post dose at Week 4 or Week 8

AAPPO, Alopecia Areata Patient Priority Outcomes; AE, adverse event; BRI, Behavior Regulation Index; BRIEF, Behavior Rating Inventory of Executive Function; CDLQI, Children's Dermatology Life Quality Index; CFB, change from baseline; CRI, Cognitive Regulation Index; EBA, Eyebrow Assessment; ELA, Eyelash Assessment; ERI, Emotion Regulation Index; PGI-C, Patient Global Impression of Change; PROMIS, Patient-Reported Outcomes Measurement Information System; SAE, serious adverse event; SALT, Severity of Alopecia Tool; SALT ≤10, ≤10% scalp without hair; SALT ≤20, ≤20% scalp without hair.
*Assessed at screening, baseline, and Weeks 2, 4, 8, 12, 18, and 24. †Except for primary endpoint assessment at Week 24. ‡Response based on achieving ≥2-grade improvement or an ELA score of 3 at all visits in patients with abnormal ELA at baseline. §Response based on achieving ≥2-grade improvement or an EBA score of 3 at all visits in patients with abnormal EBA at baseline. ‖Except for key secondary endpoint assessment at Week 24; PGI-C response defined as "moderately improved" or "greatly improved"; assessed at Weeks 2, 4, 8, 12, 18, and 24. ¶Assessed at Weeks 2 and 18.

Table 3. Endpoints for the LTE study of ritlecitinib in pediatric patients

Primary Endpoints for All Countries	
Safety endpoints	Incidence of treatment-emergent AEs (including audiological and neurological)
	Incidence of SAEs and AEs leading to permanent discontinuation from the study
Secondary Endpoints for All Countries	
Efficacy endpoints	CFB* in SALT score†
	SALT ≤10 response†
	SALT ≤20 response†
	ELA response†,‡
	EBA response†,§
Patient-centered outcome endpoints	PGI-C response‖
	CFB* in PROMIS Parent Proxy Depressive Symptoms T-score¶
	CFB* in PROMIS Parent Proxy Anxiety Symptoms T-score¶
	CFB* in modified CDLQI total score¶
Neuropsychological status	CFB* in BRIEF®2 T-scores for 3 index scores (BRI, ERI, CRI)¶
Cognitive development status	CFB# in WISC-V at Month 36

AE, adverse event; BRI, Behavior Regulation Index; BRIEF, Behavior Rating Inventory of Executive Function; CDLQI, Children's Dermatology Life Quality Index; CFB, change from baseline; CRI, Cognitive Regulation Index; EBA, Eyebrow Assessment; ELA, Eyelash Assessment; ERI, Emotion Regulation Index; LTE, long-term extension; PGI-C, Patient Global Impression of Change; PROMIS, Patient-Reported Outcomes Measurement Information System; SAE, serious adverse event; SALT, Severity of Alopecia Tool; SALT ≤10, ≤10% scalp without hair; SALT ≤20, ≤20% scalp without hair; WISC-V, Wechsler Intelligence Scale for Children Fifth Edition.
*For patients rolling over from the phase 3 study with ≤30 days between the last dose in that study and the first visit of the LTE, the phase 3 study baseline will be used. For patients rolling over from the phase 3 study with >30 days between the last dose in that study and the first visit of the LTE and for patients from the phase 1 study, the LTE baseline will be used. †Assessed at screening, baseline, and 3-monthly visits until Month 36. ‡Response based on achieving ≥2-grade improvement or an ELA score of 3 at all visits in patients with abnormal ELA at baseline. §Response based on achieving ≥2-grade improvement or an EBA score of 3 at all visits in patients with abnormal EBA at baseline. ‖PGI-C response defined as "moderately improved" or "greatly improved"; assessed at 3-monthly visits until Month 36. ¶Assessed at baseline and 3-monthly visits until Month 36. #WISC-V baseline: for patients who roll over from the phase 3 study and who complete the LTE study, WISC-V will be conducted at baseline in the phase 3 study and at the end of the LTE study; For patients who roll over from the phase 1 study and who complete the LTE study, WISC-V will be conducted at baseline in the LTE study and at the end of the LTE study.

CONCLUSIONS

- Ritlecitinib (50 mg QD) is approved for treatment of severe AA in adults and adolescents aged ≥12 years
- There is an unmet need for an approved treatment for younger **pediatric patients** with severe AA
- These studies will provide clinical data on the short-term and long-term efficacy, safety, and tolerability of ritlecitinib 50 mg and 30 mg in children aged 6 to <12 years with severe AA

Presented at the Winter Clinical Dermatology Conference – Hawaii®
January 16-25, 2026 | Hyatt Regency Maui, Hawaii

REFERENCES
1. Tan E, et al. *Int J Dermatol.* 2002;41:748-753.
2. Shellow WV, et al. *Int J Dermatol.* 1992;31:186-189.
3. King B, et al. *Lancet.* 2023;401:1518-1529.
4. Gonzalez ME, et al. *Pediatr Dermatol.* 2025;42:742-746.
5. King B, et al. *J Am Acad Dermatol.* 2021;85:379-387.
6. Piliang M, et al. *Br J Dermatol.* 2025;192:215-227.
7. King B, et al. Am *J Clin Dermatol.* 2024;25:299-314.
8. Hordinsky M, et al. *Pediatr Dermatol.* 2023;40:1003-1009.
9. Tziotzios C, et al. *J Eur Acad Dermatol Venereol.* 2025;39:1152-1162.

DISCLOSURES
This study was sponsored by Pfizer Inc. **BMP** has received honoraria from Pierre Fabre-Ducray, Difa Cooper, Dercos-L'Oréal, Legacy Healthcare, Pfizer, Eli-Lilly, Almirall, Olistic, and Giuliani. **JS** has been a speaker for Celgene, Regeneron/Sanofi, and Ortho Dermatologics; a speaker and investigator for Amgen, AbbVie, and Pfizer; a speaker, investigator, and advisor for Eli Lilly; an investigator and advisor for LEO Pharma; an investigator, speaker, and consultant for Novartis; an investigator for UCB, Janssen, Kyowa Kirin, KoBioLabs, and Castle Biosciences; an investigator and consultant for Dermavant; a speaker and consultant for Bristol Myers Squibb; and a speaker, investigator, and consultant for Arcutis. **TP** has received honoraria and/or consulting fees from ACM, Almirall, AbbVie, Amgen, Astellas, Beiersdorf, Bristol Myers Squibb, Caudalie, Celgene, Galderma, GlaxoSmithKline, Hyphen, Incyte, ISDIN, Isispharma, Janssen, La Roche-Posay, LEO Pharma, Lilly, L'Oréal, Merck Sharp & Dohme, NAOS, Novartis, Pierre Fabre, Pfizer, Sanofi-Genzyme, Sun Pharmaceutical Industries, SVR, Symrise, Takeda, UCB, Vichy, and VYNE Therapeutics. He is the cofounder of NIKAIA Pharmaceuticals and founder of SUNLUTION. **MK-I** is a principal investigator of clinical studies by Pfizer Inc and has received honoraria for lectures by Pfizer Inc. **MEG** is a consultant, investigator, and/or speaker for AbbVie, Abeona, Alphyn Biologics, Amgen, Anterogen, Apogee Therapeutics, Arcutis, Dermavant, Galderma, Incyte, Krystal Biotech, LEO Pharma, Lilly, Nielsen BioSciences, Novartis, Pelthos Therapeutics, Pfizer, Regeneron, Sanofi, and Verrica. **GSHR**, **APD**, **DW**, **HT**, **ML**, and **RW** are employees of Pfizer Inc and may own stock/stock options in Pfizer Inc. Third-party medical writing assistance, provided by Nucleus Global, was funded by Pfizer Inc.

Electronic Poster