Real-World Adherence and Persistence With Leniolisib in Patients With Activated Phosphoinositide 3-Kinase Delta Syndrome (APDS)

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KEY TAKEAWAYS

Adherence and persistence were high at 12 months in patients with APDS receiving leniolisib:

- 70% of patients were adherent with leniolisib
- 80% of patients were persistent with leniolisib

Fifteen of 83 patients (18%) receiving adherence counseling showed a 16% improvement in medication adherence

INTRODUCTION

- APDS is a rare inborn error of immunity associated with chronic and progressive clinical manifestations, including immunodeficiency, autoinflammatory manifestations, and recurrent infections[1-4]
- In March 2023, leniolisib was approved for the treatment of adult and pediatric patients 12 years of age and older
- Medication nonadherence is typically associated with worsening of medical conditions and decreased health outcomes.[5] Patient adherence to medications may be increased through various interventions, including counseling sessions[6]

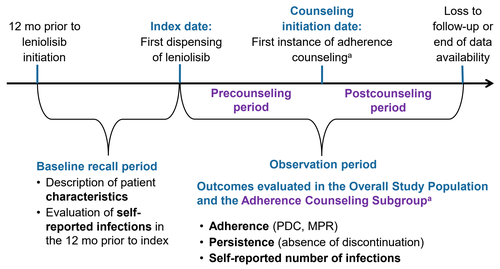
OBJECTIVE

- This study examined real-world adherence and persistence with leniolisib and the impact of adherence counseling on adherence and infection rates in patients with APDS newly initiating leniolisib treatment

METHODS

- **Study design:** Retrospective cohort following leniolisib approval using data (April 5, 2023-January 9, 2025) from PANTHERx Rare Pharmacy's proprietary specialty workflow technology (SWFT) patient engagement platform
- **Patients:** Confirmed APDS, aged ≥12 years, newly initiated on leniolisib; assessments occurred at baseline and quarterly via pharmacy records and telephone interviews (**Fig 1**); those with prior leniolisib experience were excluded
- **Outcomes assessed:**
  - Adherence and persistence
  - Self-reported infection rates
  - Impact of counseling on adherence and infection rates
- **Analyses:** Data summarized using descriptive statistics; persistence evaluated using Kaplan Meier methods; infection rate comparisons precounseling and postcounseling were conducted using rate ratios (RRs), CIs, and *P* values derived from generalized estimating equations for Poisson regression models with robust standard errors. Rates of infection, calculated as the number of events divided by the patient-years of observation, were reported per patient-year (PPY)
- This research was reviewed by the WCG institutional review board and was granted an exemption

Figure 1 Study Design



[a]Patients with a gap ≥10 days in leniolisib use in a given quarter and no infection improvements were eligible for adherence counseling.

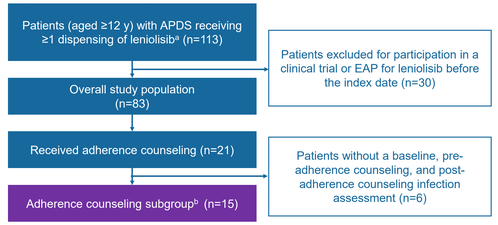
- **Proportion of days covered (PDC):** unique days with medication divided by total days of observation. Adherent was defined as PDC ≥0.8
- **Medication possession ratio (MPR):** total days' supply dispensed divided by the number of days from first fill to the end of supply from the last dispensing
- **Discontinuation:** gap of >60 days supply between dispensings, or between the last dispensing and the end of data availability

RESULTS

- A total of 83 patients were included in the study population, 15 (18.1%) of whom had been enrolled in the adherence counseling program (**Fig 2**)
- Patient characteristics were similar between the adherence population and the overall study population (**Table 1**)

RESULTS (cont)

Figure 2 Patient Disposition



[a]The first dispensing of leniolisib was the index date. [b]Patients with a gap ≥10 days in leniolisib use in a given quarter and no infection improvements were eligible for adherence counseling; eligible patients also had a baseline infection assessment, pre-adherence counseling infection assessment, and a post-adherence counseling infection assessment.
APDS, activated phosphoinositide 3-kinase delta syndrome; EAP, Expanded Access Program.

Table 1 Patient Characteristics

Characteristic	Overall study population (N=83)	Adherence counseling subgroup (n=15)
Age, median (IQR), y	21 (15-35)	21 (15-33)
Age, n (%)		
12-17 y	35 (42.2)	7 (46.7)
18-29 y	19 (22.9)	4 (26.7)
30-39 y	15 (18.1)	3 (20.0)
40-49 y	9 (10.8)	1 (6.7)
50-59 y	4 (4.8)	0 (0)
≥60 y	1 (1.2)	0 (0)
Sex, n (%)		
Female	43 (51.8)	8 (53.3)
Male	40 (48.2)	7 (46.7)
Prescriber specialty/type, n (%)		
Allergy/immunology	68 (81.9)	11 (73.3)
Pediatric	9 (10.8)	3 (20.0)
Specialist	5 (6.0)	2 (13.3)
Primary care	4 (4.8)	1 (6.7)
Hematology/oncology	2 (2.4)	0 (0)
Nurse practitioner	2 (2.4)	0 (0)
Infectious disease	1 (1.2)	1 (6.7)
Internal medicine	1 (1.2)	0 (0)
Insurance plan type, n (%)		
Medicaid	43 (51.8)	10 (66.7)
Commercial	32 (38.6)	5 (33.3)
Medicare	5 (6.0)	0 (0)
Unknown	3 (3.6)	0 (0)
Initial fill type, n (%)		
Commercial	52 (62.7)	10 (66.7)
Quick start[a]	31 (37.3)	5 (33.3)

[a]Rapid shipment of an initial 15-day supply for qualified patients.

Adherence in the Counseling Subgroup

- Fifteen of 83 (**18.1%**) patients with infection assessments (baseline, pre- and post-adherence counseling infection assessments) **received adherence counseling**
- Pre- and post-adherence counseling periods mean (SD) number of observation days was 187 (87) and 326 (147), respectively
- Adherence improved by 16% with counseling; mean (SD) PDC before and after counseling was 0.75 (0.17) and 0.87 (0.12), respectively

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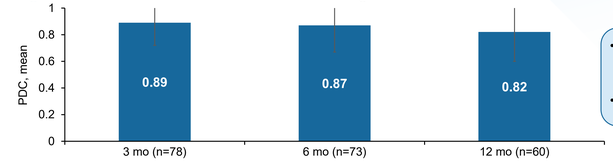
CONCLUSIONS

- **Patients initiating leniolisib for treatment of APDS demonstrated adherence and persistence to leniolisib**
  - 70% of patients with APDS achieved high adherence (PDC ≥0.8) at 12 months
  - 80% of patients with APDS remained persistent at 12 months
- Less than 1 in 5 patients (15 of 83, or 18%) received adherence counseling; among these 15 patients, counseling improved medication adherence by 16%
- As APDS is a chronic condition, adherence counseling may be beneficial to maintain or improve the ability of these patients to remain on treatment

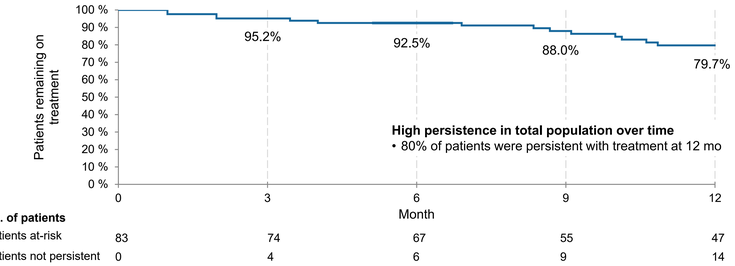
Figure 3 Overall PDC With Leniolisib Indicated High **Adherence** Over Time[a]



[a]Error bars represent SD.

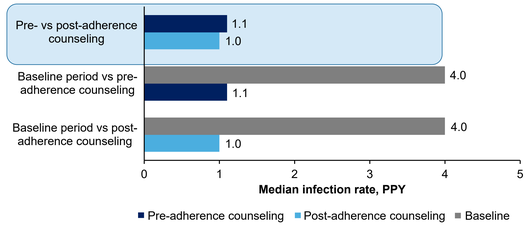
- **70% (42 of 60) of patients were adherent (PDC ≥0.8) at 12 months**
- Mean (SD) MPR was 0.91 (0.11)

Figure 4 Overall **Persistence** Was High With Leniolisib



No. of patients					
Patients at-risk	83	74	67	55	47
Patients not persistent	0	4	6	9	14

Figure 5 Effect of Counseling on Self-Reported Infection Rates (n=15)



	Rate ratio (95% CI)	*P* value
Pre- vs post-adherence counseling	0.91 (0.46-1.81)	0.792
Baseline period vs pre-adherence counseling	**0.31 (0.17-0.57)**	**<0.001**
Baseline period vs post-adherence counseling	**0.27 (0.16-0.45)**	**<0.001**

DISCUSSION

Strengths:

- Sample size is robust given the low prevalence of APDS, an ultra-rare disease
- Real-world data are reflective of the actual patient experience outside of a clinical trial setting

Limitations:

- Patients with prior leniolisib experience were excluded because complete data on their infections before and after leniolisib initiation were unavailable. Data from those patients may provide a more comprehensive understanding of the impact of leniolisib on infection and adherence rates, given their longer treatment history
- Infections were self-reported and potentially subject to recall bias

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1. Jamee M, et al. *Clin Rev Allergy Immunol.* 2020;59(3)323-333. **2.** Büsch K, et al. *Adv Ther.* 2025;42(2):752-771. **3.** Coulter TI, et al. *J Allergy Clin Immunol.* 2017;139(2):597-606.e4. **4.** Poli MC, et al. *J Hum Immun.* 2025;1(1):e20250003. **5.** Aremu TO, et al. *Pharmacy (Basel).* 2022;10(5):106. **6.** Kini V, Ho PM. *JAMA.* 2018;320(23):2461-2473.

DISCLOSURES

MEMY is a speaker/consultant for Pharming Healthcare, Inc., Amgen, and Koru Pharma. JK and EB are employees of PANTHERx Rare Pharmacy, which received consulting fees from Pharming Healthcare, Inc. SM, GG, CS, and FL are employees of Groupe d'analyse SRI, which received consulting fees from Pharming Healthcare, Inc. NCP is a speaker for Amgen, Pharming Healthcare, Inc., X4 Pharmaceuticals, ADMA Biologics, Grifols, and Takeda; performs research for Takeda and ACAAI; and serves on data monitoring committees for Janssen and Takeda. JF received consulting fees from Pharming Healthcare, Inc. AH is an employee of Pharming Healthcare, Inc.

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