Long-term safety and efficacy data with inhaled pirfenidone (AP01) in the ATLAS open-label extension study up to 240 weeks

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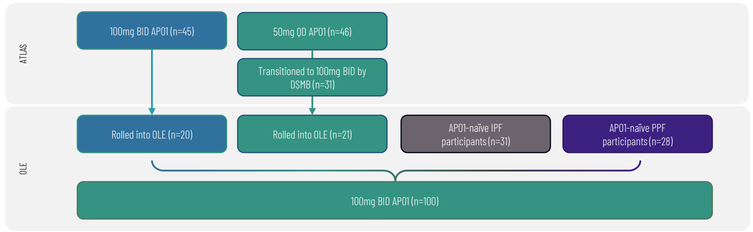
BACKGROUND

- AP01 is a novel formulation of pirfenidone, an FDA approved oral drug. AP01 is optimized for inhalation using the PARI eFlow® nebulizer (see right).
- The safety, tolerability and efficacy of AP01 was previously studied in idiopathic pulmonary fibrosis (IPF) and is currently being evaluated in progressive pulmonary fibrosis (PPF) in the double-blind, randomized Phase 2b MIST study (NCT06329401).[1]
- The ATLAS study of AP01 in IPF began in June 2019 and was followed by an open-label extension (OLE) study.[2]
- Additional treatment naïve study participants with IPF and PPF were enrolled in the OLE study.
- We report AP01 safety and efficacy in patients with up to 240 weeks follow up.

Image of the PARI eFlow nebulizer used to administer AP01. Vibrating mesh membrane delivers 8 mL over a median time of 8-9 minutes; particle size optimized to reach the alveoli.

ATLAS AND OPEN-LABEL EXTENSION STUDY DESIGN

- The ATLAS study was open-label, meaning not blinded. Patients living with IPF, as defined by ATS/ERS guidelines, were randomized to receive AP01 50 mg daily (QD) or 100 mg twice daily (BID) for 24 weeks.[3]
- The Data and Safety Monitoring Board advised that all 50 mg QD participants were to be transitioned to 100 mg BID
- All cohorts in the OLE study received 100 mg BID AP01 throughout.
- Forced Vital Capacity (FVC), a measure of lung function assessed by spirometry, was measured and recorded every 3 months

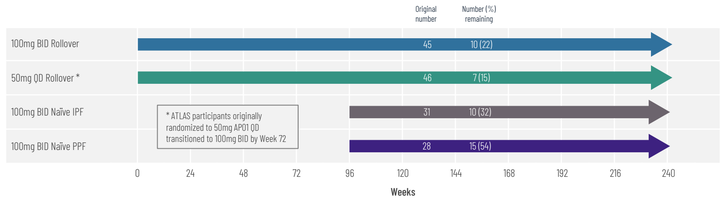


ATLAS OLE as of October 15, 2024 : n=42

ATLAS OPEN-LABEL EXTENSION PARTICIPANTS

- IPF and PPF were defined by the local investigators according to international guidelines.
- 17/91 ATLAS participants remained on AP01 at 240 weeks.
- 10/31 AP01-naïve IPF participants and 15/28 AP01-naïve PPF participants had reached week 144 on AP01 (data cut-off Oct 15, 2024).

ATLAS OLE Participant Composition



CONCLUSIONS

- Up to 240 weeks follow up, AP01 appears to have continuing efficacy and a stable safety profile with long-term open-label treatment.
- This data provides support for the ongoing evaluation of AP01 in the Phase 2b MIST study and a future Phase 3 study.

The Phase 1b ATLAS study was funded by Avalyn Pharma Inc.

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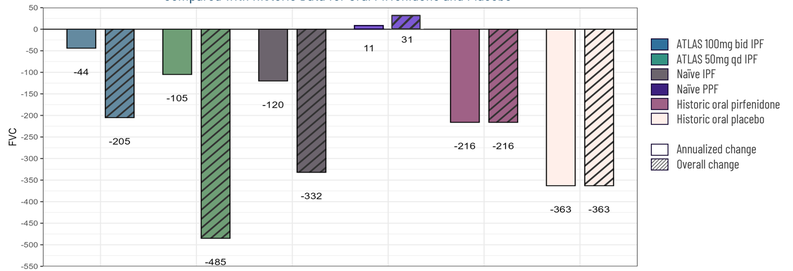
ATLAS OLE RESULTS - EFFICACY

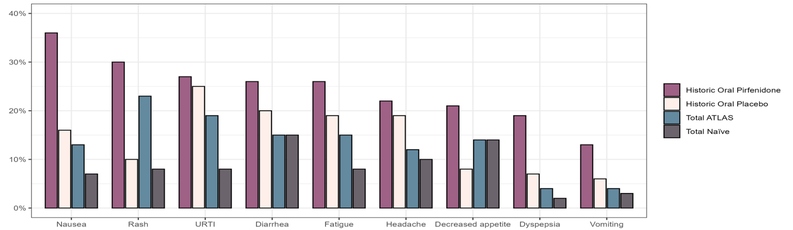
Forced Vital Capacity (FVC) in ATLAS OLE Participants (Rollover and AP01-Naïve) Compared with Historic Data for Oral Pirfenidone and Placebo



- Roll-over patients from ATLAS study had an FVC change of -69 ml/year; naïve IPF patients had a FVC change of -120 ml/year while naïve PPF patients had a FVC change of +11 ml/year.
- By comparison the annual change in FVC in the pooled CAPACITY and ASCEND studies was -216 ml/year for oral pirfenidone and -363 ml/year for placebo.[4]

ATLAS OLE RESULTS - SAFETY

Adverse Event Frequencies in ATLAS OLE Participants (Rollover and AP01-Naïve) Compared with Historic Data for Oral Pirfenidone and Placebo



- Adverse events were present in 95.3% of participants overall, demonstrating similar frequencies across cohorts.
- No new safety signals were demonstrated.

References

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2. Australian New Zealand Clinical Trials Registry (ANZCTR). 2025 [cited October 13, 2025] Available from https://anzctr.org.au/Trial/Registration/TrialReview.aspx?ACTRN=12618001838202
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Abbreviations: OLE: open-label extension; FVC: forced vital capacity; IPF: idiopathic pulmonary fibrosis; PPF: progressive pulmonary fibrosis; ATS: American Thoracic Society; ERS: European Respiratory Society; QD: once daily dosing; BID: twice daily dosing