Access to Pirfenidone Solution for Inhalation (AP01) for Treatment of Progressive, Fibrosing Interstitial Lung Diseases, including Idiopathic Pulmonary Fibrosis

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This study has been transitioned to CTIS with ID 2024-515964-30-00 check the CTIS register for the current data. • Allow patients to continue or start AP01 therapy for the treatment of ILD and IPF prior to regulatory approval or until the study is...

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Respiratory disorders NEC
Study type	Interventional

Summary

ID

NL-OMON52355

Source
ToetsingOnline

Brief title
AP01-005

Condition

- Respiratory disorders NEC

Synonym
Interstitial Lung Disease (ILD) which cause inflammation and scarring (firbrosis) of the lungs

Research involving
Human

Sponsors and support

Primary sponsor: Avalyn Pharma, Inc
Source(s) of monetary or material Support: Avalyn Pharma;Inc.

Intervention

Keyword: continue or start treatment, Fibrosing ILD, Idiopathic Pulmonary Fibrosis, Pirfenidone Solution for Inhalation (AP01), Progressive

Outcome measures

Primary outcome

Safety Outcome Measures

• Treatment-emergent adverse events (AEs)

• Treatment-emergent deaths

Efficacy Outcome Measures

• No efficacy outcome measures

Secondary outcome

N/A

Study description

Background summary

The term Interstitial Lung Disease (ILD) encompasses a large group of over 200 pulmonary disorders, most of which are classified as rare. While idiopathic pulmonary fibrosis (IPF) is the classic fibrosing ILD, clinical data suggest that there is a larger group of patients with differing clinical ILD diagnoses who develop a progressive fibrosing phenotype during the course of their disease.

Momentarily the study drug (pirfenidone) is available as treatment for idiopathic pulmonary fibrosis, which is given in a capsule for oral use. However, studies have shown the use of oral pirfenidone leads to side effects

in many patients. In some cases these side effects prevent the use of medication. A previous study has shown that a single dose up to 100 mg/mL given as an inhaled formulation was safe and well tolerated. It has shown that more pirfenidone was absorbed in the lungs. This may lead to better efficacy as well as a reduction in the level of study drug which was absorbed by the blood. This causes less side effects.

Study objective

This study has been transitioned to CTIS with ID 2024-515964-30-00 check the CTIS register for the current data.

• Allow patients to continue or start AP01 therapy for the treatment of ILD and IPF prior to regulatory approval or until the study is terminated
• To evaluate safety outcomes of patients on AP01 therapy

Study design

This is a study for Pirfenidone Solution for Inhalation (AP01) 100 mg twice daily. The primary objective is to allow patients to continue or start AP01 therapy for treatment of ILD, including IPF.
A screening visit will be performed if the patient has not previously been on an Avalyn sponsored AP01 study. If a patient has been previously enrolled on an Avalyn sponsored study with AP01, a modified screening visit will occur. Patients will have an in-clinic visit every 12 weeks and AEs and lung function will be captured. If the patient stops treatment for any reason, an end of treatment visit will take place.

Intervention

Patients will receive 100 mg AP01 administered twice daily with the PARI eFlow® nebulizer

Study burden and risks

Please refer to the study schedule in the protocol, table 5 (page 36).

This study will last about 48 months but could also end sooner once the study drug obtains regulatory approval.

The subject will need to come to the hospital every 12 weeks while taking part in this study. In addition the subject is asked to come to the hospital for an end of study visit once they have completed the study drug treatment.

The screening visit (if required) and the first visit in the treatment period will last approximately 3 to 4 hours. All other visits will last approximately

1 to 2.5 hours.

During these visits the following tests and procedures will take place:
- Demographic and medical history (1 time)
- Physical exam, vital signs (7 times)
- Urine tests (3 times)
- Pregnancy tests in wo men of childbearing potential (7 times)
- Blood test (7 times)
- Spirometry tests (7 times).

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)

Inclusion criteria

A patient must meet the following inclusion criteria to be eligible for enrollment in the clinical study:

1. Prior participant in an Avalyn AP01 study, excluding normal healthy volunteers
OR
2. Patients with no other treatment options with ILDs including but not limited to IPF, fibrosing phenotype ILD, pulmonary involvement of scleroderma, rheumatoid lung and silicosis. Prior Sponsor (Avalyn Pharma, Inc.) approval of non-Avalyn study rollover patients is required.
3. Diagnosed chronic progressive fibrotic lung disease, including IPF, without treatment alternatives such as
a. Not eligible for oral pirfenidone and nintedanib due to national formulary restrictions or lack of applicable regulatory approval
b. Intolerant to oral pirfenidone and nintedanib, if previously offered
c. Not eligible for an ongoing clinical study of AP01 other than this study
4. Age greater than 18 years at Screening
5. Able to understand and sign, prior to study entry, a written informed consent form (ICF) consistent with International Council on Harmonisation Guideline for Good Clinical Practice (ICH-GCP) and local laws
6. Able to understand the importance of adherence to study treatment and the study protocol and willing to follow all study requirements, including the concomitant medication restrictions, throughout the study
7. Females of childbearing potential must use an effective contraceptive method during the clinical study and 30 days after the last dose of AP01

Exclusion criteria

The presence of any of the following exclusion criteria excludes a patient from study enrollment:

Disease-Related Exclusions
1. Significant clinical worsening of IPF/ILD between Screening and Day 1, in the opinion of the Investigator
2. Not a suitable candidate for enrollment or unlikely to comply with the requirements of this study, in the opinion of the Investigator
3. History of acute IPF exacerbation requiring hospitalization in the last 30 days
4. Clinical evidence of active infection, including but not limited to bronchitis, pneumonia, sinusitis, urinary tract infection, or cellulitis
Medical Exclusions
5. Females with a positive pregnancy test at Screening or are currently breastfeeding
6. Any history of malignancy likely to result in significant disability or likely to require significant medical or surgical intervention within the next 6 months. This does not include minor surgical procedures for localized cancer (e.g., basal cell carcinoma)
7. Any condition other than IPF that, in the opinion of the investigator, is

likely to result in the death of the patient within the next 6 months
8. History of severe hepatic impairment or end-stage liver disease or AST or ALT greater than 5 times the upper limit of normal at Screening
9. History of end-stage renal disease requiring dialysis
10. Participation in a clinical study with administration of an investigational drug product within the previous 30 days, or five half-lives of the previously administered investigational product
11. Hypersensitivity to the active substance or to any of the excipients of pirfenidone

Study design

Design

Study phase:	2
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	16-07-2021
Enrollment:	4
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Pirfenidone Solution for Inhalation
Generic name:	N/A

Ethics review

Approved WMO

Date:	07-01-2021

Application type:	First submission
Review commission:	MEC-U: Medical Research Ethics Committees United (Nieuwegein)
Approved WMO Date:	21-04-2021
Application type:	First submission
Review commission:	MEC-U: Medical Research Ethics Committees United (Nieuwegein)
Approved WMO Date:	09-03-2022
Application type:	Amendment
Review commission:	MEC-U: Medical Research Ethics Committees United (Nieuwegein)
Approved WMO Date:	21-02-2024
Application type:	Amendment
Review commission:	MEC-U: Medical Research Ethics Committees United (Nieuwegein)
Approved WMO Date:	05-03-2024
Application type:	Amendment
Review commission:	MEC-U: Medical Research Ethics Committees United (Nieuwegein)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
Other	380951
EU-CTR	CTIS2024-515964-30-00
EudraCT	EUCTR2020-005103-39-NL
CCMO	NL75957.100.20