

PATIENT CASE SERIES SUMMARY:

Treating autoimmune PAP Long-term with Molgramostim Improved Lung Function, Symptoms, and Quality of Life in 5 Patients

What is autoimmune PAP?

Autoimmune pulmonary alveolar proteinosis (autoimmune PAP) is a rare lung disease. Surfactant normally helps keep the air sacs in lungs open. In people with autoimmune PAP, surfactant builds up and makes it hard to breathe. Researchers believe molgramostim helps to activate the cells that help clear excess surfactant from the lungs. This improves oxygen flow from the lungs to the bloodstream. Currently, there are no approved medications for autoimmune PAP in the United States or Europe. However, the symptoms of autoimmune PAP can be treated with whole lung lavage. Whole lung lavage is an invasive procedure, performed under general anesthesia, that temporarily removes surfactant.

What is a patient case series?

In a patient case series, researchers collect information and provide outcomes about a small number of patients who have undergone the same treatment for a specific disease.

What was the focus of this patient case series on molgramostim?

The focus was to report the long-term outcomes of treatment with molgramostim in 5 European patients with autoimmune PAP and whether treatment helped improve lung function and reduce autoimmune PAP symptoms. For this case series, researchers reported on:

The health and progress records of 5 European adults living with severe autoimmune PAP who were treated with molgramostim for 2.5 to 5.5 years

All 5 adults were treated with molgramostim inhalation solution through a compassionate use program, which is a way patients with a life-threatening disease are provided with a medicine that is not yet approved

What was measured?



Lung function: Diffusing capacity of the lungs for carbon monoxide (DLCO). This measures how well lungs move gas, like oxygen, from inhaled air to the bloodstream.



Surfactant build up: Measured using CT scans taken before and after molgramostim treatment.



Serious adverse events: A health issue that occurs during a medical treatment, study, or procedure and can lead to hospital care, lasting medical problems, life-threatening conditions, or death.



Quality of life: Patient reports on changes in symptoms, participation in daily life and use of supplemental oxygen.

What was found?



Lung function: 5 out of 5 patients had improved lung function.



Surfactant build up: CT scans showed surfactant build up reduced in 5 out of 5 patients. Improvement was observed as early as 10–12 months of being on molgramostim.



Serious adverse events: Molgramostim was well tolerated; zero patients reported serious adverse events.



Quality of life: 5 out of 5 reported improved symptoms and quality of life.



And more: Zero patients needed whole lung lavage after at least one year of treatment with molgramostim. 4 patients reduced or stopped their use of supplemental oxygen.

Where can I learn more about aPAP?

Visit www.SavaraPharma.com to learn more.

Where can I learn about the patient case series?

Visit [ERJ Open Research](#) and search the title: “Long-Term Outcomes in Five Patients with Autoimmune Pulmonary Alveolar Proteinosis Treated with Molgramostim Inhalation Solution”

People living with autoimmune PAP should consult their physicians with questions about their individual care and should not make changes in their treatments based on the results of this research.

