

CASE REPORT: INHALED GRANULOCYTEMACROPHAGE COLONY-STIMULATING FACTOR FOR MILD-TO-MODERATE AUTOIMMUNE PULMONARY ALVEOLAR PROTEINOSIS – 24 MONTHS OF FOLLOWUP

I. Oterino Moreira¹, I. Morona Mínguez², MJ. Linares Asensio³, S. Sanz Márquez¹, M. Pérez Encinas¹

1. Pharmacy Department. Alcorcon Foundation University Hospital Spain, 2. Pharmacy Department. Mostoles University Hospital. Spain, 3. Pulmonology Department. Alcorcon Foundation University Hospital. Spain

AIM AND OBJECTIVES

Autoimmune pulmonary alveolar proteinosis (aPAP) is a disease caused by IgG antibodies against granulocyte-macrophage colony-stimulating factor (GM-CSF). The treatment of choice is bronchoalveolar lavage (BAL) to remove the lipopotenaceous material accumulated by the poor clearance of alveolar surfactant.

BACKGROUND AND IMPORTANCE

To present the clinical, radiological and functional evolution during 24 months of treatment with the experimental therapy inhaled GM-CSF in aPAP

MATERIALS AND METHODS

A 37-year-old male diagnosed with aPAP in April 2014 who has required 3 BAL (October 2014, February 2016 and March 2019). In the last BAL, the patient developed major complications that required admission to the intensive care unit. In October 2019, he presented a new worsening (see table and figure), so off-label treatment with recombinant human sargramostim GM-CSF (250µg inhaled every 12 hours for 7 days every other week) was chosen.

The pharmacy service prepared a favorable report on off-label treatment and requested drug from regulatory agency. In the pharmaceutical care consultation, the administration technique, stability, dosage regimen, storage (2-8°C) were explained and doubts were resolved.

RESULTS

Clinical and functional evolution of the ventilatory parameters and the six-minute walk test (6MWT).

	7 months after third BAL	3 months treatment	6 months treatment	18 months treatment	24 months treatment
Pulmonary function test					
FEV1, ml (%)	2630 (63)	2690 (72)	3080 (75)	2900 (71)	3030 (75)
cTLCO (%)	37	53	52	74	68
cKCO (%)	59	69	64	85	74
6MWT					
Theoretical distance (m)		614.52	435.71	627.06	619.17
Heart rate minute 6 (beats per minute)		136	108	108	105
Oxygen saturation minute 6 (%)		89	89	93	91



Chest X-ray evolution

1. Before third BAL
2. Worsening after 7 months after third BAL
3. Improvement after 3 months of treatment
4. Stability after 18 months of treatment

After 24 months of treatment, the patient has not presented any adverse events and maintains an excellent clinical, functional and radiological response with significant improvement in gas exchange, which has allowed home oxygen therapy to be withdrawn.

CONCLUSION AND RELEVANCE

Our case supports that inhaled GM-CSF has been safe and effective in the treatment of aPAP and represents a therapeutic option after resistance or contraindication to BAL.

