ORPHAZYME

Dr Harald Enzmann
European Medicines Agency
Domenico Scarlattilaan 6
1083 HS Amsterdam
The Netherlands

Copenhagen, March 22, 2022

Subject: Withdrawal of for Miplyffa (arimoclomol citrate) 31 mg, 47 mg, 62 mg, 93 mg and 124 mg hard capsules - EMA product reference: EMEA/H/C/005203

Dear Dr Enzmann,

I would like to inform you that Orphazyme A/S has taken the decision to withdraw the application for Marketing Authorisation of Miplyffa (arimoclomol citrate) 31 mg, 47 mg, 62 mg, 93 mg and 124 mg hard capsules, which was intended to be used for the treatment of Niemann-Pick disease type C (NPC).

This decision was taken because the CHMP considers that the data provided do not allow the committee to conclude on a positive benefit risk balance, and the Applicant cannot address the major objection raised by the CHMP within the framework of this MAA procedure.

The withdrawal does not have any impact on ongoing clinical trials and compassionate use programmes.

We reserve the right to make further submissions at a future date in this or other therapeutic indication(s).

I agree for this letter to be published on the European Medicines Agency website.

Yours sincerely,