DEPARTMENT OF HEA' 'H & HUMAN SERVICES

Public Health Service

Food and Drug Administration
Rockville MD 20857

August 12, 1997

225-97-8003

Mr. Terry Slater
National Manager
Therapeutic Goods Administration
Commonwealth Department of Health
and Family Services
PO Box 100
Woden ACT 2606
AUSTRALIA

Dear Mr. Slater:

The U.S. Food and Drug Administration is pleased to cooperate with your government in facilitating the exchange of documents and information concerning a drug or biological preparation that your government is considering for orphan product status. We hope that this cooperation will facilitate and expedite access to needed therapy for Australian patients with rare diseases.

Upon request from the Australian Therapeutic Goods Administration (TGA), and to the extent permitted by U.S. law and FDA regulations, and as appropriate, with the permission of the U.S. sponsor, the FDA Office of Orphan Products Development (OPD) intends to provide to the TGA a copy of the U.S. designation request and review performed by the OPD on a particular product, whether such orphan designation request has or has not been granted..

Upon request from the TGA, and under the same terms and conditions noted in the preceding paragraph, the Center for Drug Evaluation and Research (CDER) or the Center for Biologic Evaluation and Research (CBER) intends to provide summary information concerning evaluation and approval of a particular product. OPD expects to be receptive to requests for assistance in seeking permission of the sponsor to permit TGA to utilize the necessary information. We understand that the reports and information FDA provides will form the basis for a similar orphan product evaluation for Australia.

Information provided by FDA pursuant to this arrangement will be provided in confidence to the TGA. The information will be provided in accordance with FDA law and regulations, including privacy and confidentiality requirements, and with assurances of TGA's authority and commitment to protect the information from public disclosure in Australia. Copies of designation or evaluation reports will be provided to the TGA in conformance with the requirements of Part 20 of Title 21, U.S. Code of Federal Regulations, and with the written consent (where appropriate) of the U.S. sponsor of the designation request or product approval application.

For the purpose of coordination, we propose that the respective liaison officials be:

For the FDA: 225-97-8003

Director, Office of Orphan Products Development
Food and Drug Administration/HF-35
5600 Fishers Lane Room 8-73
Rockville, Maryland 20857
U.S.A.
Telephone: 301 827-3666
FAX: 301 443-4915

For the Australian TGA:

Director, Drug Safety and Evaluation Branch
Therapeutic Goods Administration
P.O. Box 100
Woden, ACT 2606
Australia
Telephone: 61 2 6232 8100
FAX: 61 2 6232 8140

To help ensure that this information exchange program works well and meets our mutual needs and requirements, we feel that it is important that, at appropriate intervals, and by mutual concurrence, a discussion or meeting take place between representatives of our two agencies to assess the activities and the provisions outlined in this letter.

We anticipate that these arrangements will provide a sound basis on which further cooperative arrangements between us on products for patients with rare diseases will develop.

Sincerely,

Marlene E. Haffner, M.D., M.P.H.
Rear Admiral, United States Public Health Service
Director, Office of Orphan Products Development

TGA | Therapeutic Goods Administration

PO Box 100 Woden ACT 2606 Australia
Telephone: (06) 232 8444. Fax: (06) 232 8605

Commonwealth Department of
Health and
Family Services

225-97-8003

Marlene E. Haffner MD, MPH
Rear Admiral, United States Public Health Service
Director, Office of Orphan Products Development
5600 Fishers Lane, HF-35
Room 8-73
ROCKVILLE MD 20857 USA

Dear Dr Haffner

The purpose of this letter is to formalise our agreement regarding provision of information on orphan drugs by the U.S. Food and Drug Administration (FDA) U.S.A. to the Therapeutic Goods Administration (TGA), Department of Health and Family Services, Australia.

The TGA formally requests that the U.S. FDA provide orphan drug designation reports and orphan drug evaluation reports to the TGA.

In the spirit of co-operation, and on behalf of the TGA, I agree as follows:

1. Following receipt by the TGA of an application requesting orphan drug designation of a drug in Australia, the TGA will request from the Office of Orphan Drugs Development a copy of an orphan drug designation report for the drug.

This request will apply in cases where the drug has been granted orphan drug designation in the U.S. or where the drug has been refused orphan drug designation in the U.S.

2. Following receipt by the TGA of an application to register a product for which orphan designation has been granted for the drug in Australia, the TGA will request from the U.S. FDA a copy of the Center for Drug Evaluation and Research (CDER) evaluation report or the Center for Biologics Evaluation and Research (CBER) evaluation report.

This request will apply in cases where a drug has been granted orphan drug designation in the U.S. and where an application to register a product containing that drug in the U.S. has been approved, refused or is pending.

3. It is intended that where possible, the reports provided under this arrangement will form the basis of the evaluation of similar applications in Australia. Therefore, the reports must be sufficiently complete to enable appropriate evaluation of the product.

4. Information will be provided in accordance with agency regulations (including confidentiality requirements).

5. Copies of designation reports or evaluation reports will be provided to the TGA only after the written consent of the U.S. sponsor of the designation request or product registration application has been obtained, except as otherwise provided in FDA's regulations disclosure (21 CFR 20.89).

6. Liaison officers for the purpose of coordinating these provisions are as follows:

A. For FDA:

Director, Office of Orphan Products Development
Food and Drug Administration
5600 Fishers Lane, HF-35
Rockville, Maryland 20857
U.S.A.
Telephone: 1 301 827 3666
Facsimile: 1 301 443 4915

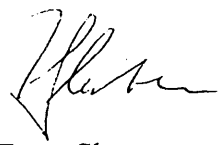
B. For TGA:

Director, Drug Safety and Evaluation Branch
Therapeutic Goods Administration
PO Box 100
Woden, ACT 2606
Australia
Telephone: 61 2 6232 8100
Facsimile: 61 2 6232 8140

I am confident the implementation of these provisions will provide a sound basis on which to develop further cooperative arrangements between us on orphan drug products and to work toward a reciprocal arrangement in the future.

I look forward to your official confirmation these arrangements can be agreed.

Yours sincerely



Terry Slater
National Manager
Therapeutic Goods Administration

12 August 1997