

# MAPS Succeeds in Securing an Orphan Drug Designation for Marijuana

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TWO AND A HALF YEARS AGO, MAPS submitted an application to the Food and Drug Administration's Office of Orphan Drug Products requesting that marijuana be designated an Orphan Drug for the treatment of AIDS wasting syndrome. The Orphan Drug program was created by Congress to facilitate development of drugs for rare diseases, defined as fewer than 200,000 patients per year. Drugs for such rare diseases have not been considered sufficiently profitable for pharmaceutical companies and research has been minimal, leaving drugs for rare diseases "orphans." Orphan Drug designation provides a package of incentives for research and development, culminating in seven years of patent protection (exclusive right to market) should convincing data about safety and efficacy ever be submitted to the FDA and the drug be approved for marketing. Marinol, the oral THC pill, was approved for AIDS wasting under the Orphan Drug program.

On May 25, 1999, the FDA wrote a letter announcing that MAPS' application, which had already been rejected five times and each time sent back to MAPS for a response, had finally been accepted. This designation is a demonstration of good faith on the part of the FDA and represents a major milestone in MAPS' efforts to support research into the medical uses of marijuana.

As MAPS members and other readers of the *Bulletin* probably know, MAPS began working in 1992 with Dr. Donald Abrams, UC San Francisco, in an effort to obtain permission for his study of the use of marijuana in AIDS patients. Dr. Abrams' study was eventually approved, received a \$970,000 grant from NIDA, and treated its first subject in 1998. Dr. Abrams' study is the first FDA-approved investigation of the medical use of marijuana in a patient population in 15 years. Dr. Abrams will complete the dosing phase of the study in early 2000. MAPS' successful effort to have marijuana declared an Orphan Drug will make it easier to continue to research the medical use of marijuana for AIDS wasting syndrome, if the data gathered by Dr. Abrams demonstrates that marijuana can be administered safely to AIDS patients.

Another benefit of having marijuana declared an Orphan Drug by the FDA is that it will theoretically be easier for MAPS to obtain a DEA license to establish a

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domestic medical marijuana production facility to produce high-quality marijuana for FDA-approved research. In late July 1999, MAPS received a grant of \$20,000 from Peter Lewis to explore whether it really will be possible to obtain a DEA license to establish a domestic medical marijuana production facility. MAPS is currently searching for the appropriate personnel with whom to prepare an application to the DEA, preferably partnering with an already existing botanical medicine company with expertise in developing plant-based medicines for pharmaceutical research.

MAPS is the first organization to use the Orphan Drug program to help facilitate research into any medical use of marijuana. The way the program is structured, other entities can also seek Orphan Drug designation, either for AIDS wasting or for other rare diseases that marijuana may be useful in treating. Congress intended Orphan Drug designation to be the starting point for scientific research that would culminate in an informed decision regarding the potential safety and efficacy of each designated drug for the treatment of the specific indication so designated.

The growth of political support for scientific research into the medical use of marijuana has resulted in several for-profit firms initiating research into various forms of marijuana extracts and isolated cannabinoids administered in non-smoking delivery systems such as vaporizers and aerosol sprays. MAPS supports all of these efforts to develop needed medicines for patients. MAPS' work on behalf of research into the plant itself is intended to ensure that an accurate risk/benefit ratio can be determined for the use of marijuana when smoked or used in a vaporizer. Profit-making firms with obligations to shareholders have powerful incentives to abandon efforts to study and obtain approval for the plant itself, which is likely to be the least expensive, least profitable dosage form and is certainly the most politically controversial. As a non-profit organization, MAPS does not face such pressure and will therefore focus on facilitating research with the marijuana plant.

Orphan Drug designation is merely the first step in a very long process. Whether any additional progress will be made remains to be determined. •