HOW SHOULD ONE MANAGE EMERGENCY DRUG REQUESTS AND THEIR DATA?

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1. INTRODUCTION

The Food and Drug Administration is aware that there may be good reason to use an investigational drug in patient care, i.e., not in a formal investigation, before complete data on effectiveness and safety are available. The usual reason is the combination of a patient with a life-threatening disease who has exhausted all standard therapy and the availability of a promising new agent with some evidence of usefulness in the condition. A request for use of a drug in such cases is usually called a request for "emergency" or "compassionate" use. Patients with life-threatening cardiac arrhythmias provide a particularly common source of these requests, and the gravity of the situation often requires quick action. The problem is thus not only to obtain permission to treat the patient with the experimental compound, but to find out how the drug can be obtained in as short a time as possible.

Emergency uses of drugs are a source of some discomfort to both FDA and the pharmaceutical industry: to FDA because these uses cannot be closely monitored and because information about the drug is incomplete; to industry because very sick patients may die or have adverse events and raise troubling questions about the drug. Nonetheless, FDA and the pharmaceutical industry have always felt that a seriously ill patient cannot be denied that medication which may offer a reasonable possibility
of benefit. It is essential however, that physicians using drugs under these circumstances carry out their obligations to their patients, to the drug manufacturer, and to FDA by monitoring patients closely and supplying needed information.

There are established procedures in our Division and in the rest of the Bureau of Drugs, for considering emergency request promptly. If an emergency occurs during non-business hours there are ways to contact the appropriate individuals, but this is a more difficult and every effort should be made to reach us between 8:00 a.m. and 5:30 p.m.

Emergency use of a drug can be carried out either under an emergency protocol developed by the drug manufacturer, if such a protocol exists, or under an application by the treating physician. Most antiarrhythmic drugs, because of their nature, do have existing emergency protocols including entry criteria, monitoring requirements, etc. Whenever possible patients will be treated under such protocols, rather than under a separate individual investigator application, as it is far easier for us and it keeps all the data together. In addition, the investigator usually need contact only the manufacturer to use the drug under these protocols.

A summary of the protocols available for commonly used investigational antiarrhythmic agents is provided below. In addition, there are the names of the appropriate individuals to contact in each case. These protocols may be amended or terminated and the individuals named may also change, but the information should be of help as a starting point.

2. MECHANISMS FOR OBTAINING INVESTIGATIONAL (NON-APPROVED) ANTIARRHYTHMIC AGENTS

2.1 Does an emergency protocol exist?

Figure I illustrates the procedure for obtaining permission to use an investigational agent in an emergency situation.

Once a physician concludes that his patient needs an investigational agent, he should contact either the drug manufacturer or