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# Clinical Trials—The Crucial Tool in the Search for a Cure

How do advances in treatment for serious diseases—from heart disease and cancer to aplastic anemia, MDS and PNH—come about? Drugs and procedures thought to hold promise must be tested, first on animals, then on human beings. Clinical trials—tightly-controlled studies of the effectiveness of new treatments—are the engine that drives medical progress. New therapies cannot be approved by the Food and Drug Administration for use in the general public until they undergo rigorous testing in clinical trials.

“Clinical trials are where the ultimate cure for bone marrow disorders will come from,” says Bob Carroll, president of the Aplastic Anemia & MDS International Foundation. Neil Young, MD, Chief of Hematology at the National Heart, Lung and Blood Institute agrees. “Without clinical trials there really is no way to learn more about how to treat a disease. For findings to be valid, a clinical trial needs a certain number of enrollees, thus making the involvement of patients with rare diseases all the more crucial.”

Clearly, high patient enrollment in clinical trials is important to medical progress and in the best interest of society. How do individual patients determine whether such enrollment is in their personal best interest? It is of primary importance to understand how clinical trials work in order to evaluate the risks and benefits of participating in any particular trial. For example, clinical trials are conducted in phases, and the phase of a given study may influence a patient’s level of comfort when considering enrollment. In Phase I studies, new drugs or treatments are used in people for the first time in order to evaluate safety and identify side effects. Phase II studies continue to evaluate safety but also look at effectiveness of the new treatment or drug. Phase III studies test the new therapy against current, standard

therapies to compare effectiveness and side effects. Phase IV studies continue to collect information about the therapy after it has been FDA approved and marketed.

“Patients’ biggest fear when considering participation in a clinical trial is the unknown,” says Ron Paquette, MD, a hematologist at UCLA. “Patients should become as educated as possible about all available therapies before embarking on a clinical trial so that they can make an informed decision. Aplastic anemia patients have several conventional treatment options and would generally consider experimental therapies only after exhausting standard approaches,” he advises. Studies are often an attractive option for MDS patients because they have fewer standard therapies to choose from. They may have to rely on experimental procedures for treatment. “The exciting thing,” Paquette adds, “is that I’ve never seen as many trials available for MDS patients as there are now.”

Patients should be aware that there are many safeguards in place to protect participants in clinical trials. Clinical trials are usually conducted at top medical centers by physicians with special expertise in the disease being studied. Independent committees at these medical centers called Institutional Review Boards (IRBs) evaluate proposed clinical trials to make certain that any risks posed to patients are offset by potential benefits. IRBs are partially responsible for making certain that clinical trials adhere to high ethical standards and that patient rights are protected.

Another safeguard that patients have when participating in a clinical trial is the FDA requirement of informed consent. Patients must be told exactly how the study will be conducted, the possible risks and benefits that exist with the therapy, and how the therapy being studied

compares with existing treatments for their disorder. Furthermore, patients always have the option of leaving a clinical trial at any time and for any reason, without compromising their future medical care.

Marilyn Baker, Executive Director of the AA&MDSIF, feels that clinical trials are often a very good option for people with AA, MDS and PNH. “I have known many patients who are alive today thanks to the excellent treatment they received participating in a clinical trial. Patients have told me that the care they received in a trial was above and beyond their local hospital experience because the health care professionals were so knowledgeable about the disease and were very dedicated to making sure that everything was administered correctly and all information is well documented. There is also the added advantage of many clinical trials offering free treatment.

There are many resources available to patients who are considering participation in a clinical trial. For general information on what clinical trials are, how they are conducted and what questions patients should ask before participating, go to the National Library of Medicine’s website: [www.ClinicalTrials.gov](http://www.ClinicalTrials.gov). For those who do not have internet access, this organization can also be reached by telephone at (888) 346-3656 or (301) 594-5983.

AA&MDSIF publishes a brochure entitled “Managing Treatment Decisions” that discusses issues that patients should discuss with their physicians before considering any treatment option. Contact the AA&MDSIF to have this brochure sent to you. It is not available online.

***The AA&MDSIF also offers a listing of current clinical trials for AA, MDS and PNH. This listing is available on our website or can be postal mailed to you.***