



## How Social Workers Fit Into the Gene Therapy Puzzle

**g**ene therapy or more accurately, gene transfer research is steadily progressing from the lab to the clinical setting. Hemophilia treatment center (HTC) social workers may wonder where they fit in. There are multiple opportunities for social workers to contribute to gene transfer research in ways that enhance both the patient's and the research team's experiences.



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## RECRUITMENT AND ENROLLMENT

Typically, HTC physicians and research nurses review eligibility criteria for gene transfer studies and determine which of their patients meet these criteria. The physician then notifies these patients that they are eligible for a specific study, describes the study and asks them to consider participating in it.

Here is an opportunity for social workers to collaborate with HTC research teams by helping them decide which patients to approach. The social worker may know that a given patient is having problems in other life areas that would interfere with his ability to adhere to a gene transfer protocol. On

the other hand, a social worker may assess that, despite stressors in other life areas, a patient is coping well and would be a good candidate for a study. Social workers are often aware of barriers that might interfere with a patient's understanding of research protocols or his ability to participate. The social worker can make the rest of the HTC team aware of these barriers and work with the patient to attempt to remove them.

In addition, social workers can assist research teams by helping them figure out how best to approach and communicate with patients about enrolling in gene transfer studies, since there are very serious issues for patients to consider. Some of the requirements for participation in gene transfer studies are life-changing, such as agreeing not to have children or deciding to bank sperm for later use, and either abstaining from sexual intercourse or using double-barrier contraceptives during the course of the study. These precautions are necessary to prevent any unforeseen problems in study participants' offspring. Social workers can be invaluable in eliciting patients' concerns related to these requirements. Despite the effort of many physicians and patients to make the physician-patient relationship more collaborative, some patients are still somewhat hesitant to express their concerns, fears and anxieties to their physician. There are several reasons for this. For example, the patient may be overwhelmed by the complexity of the study, embarrassed by some of his concerns or eager to please his physician. In such situations, the patient may feel more comfortable expressing concerns to the social worker. It is then the social worker's responsibility to empower the patient to address his concerns with the physician, or possibly to bring the two parties together and help their discussion.

## THE INFORMED CONSENT PROCESS

Typically, the HTC physician (or principal investigator) reviews the informed consent documents with patients who are considering study participation and answers any questions that the patient raises. Although "informed consent" sounds simple enough, this topic is actually quite complex and is currently a focus of research. How can a researcher be sure that a patient is *fully* informed about all the known risks of participating in a gene transfer study? How can a physician be sure that the patient *understands* all of the information that he has been given about the study? As the study proceeds, how does the researcher know that the patient *still remembers and understands* all of the information given at the beginning of the study?

This is another opportunity for social workers to become involved. The HTC social worker could sit in on the informed consent discussion to help the patient formulate and ask questions or help the physician communicate information, if needed. Social workers are trained to observe nonverbal behavior and could offer invaluable assistance, for example, by noticing a patient's unstated discomfort or anxiety and attempting to find out what the patient's concerns are.

Since gene transfer research has been going on for a longer time in cystic fibrosis than in hemophilia, it may be helpful to observe the informed consent process used in those trials. Margaret Humphries, RN, is the research coordinator for a cystic fibrosis gene transfer study at the University of Florida, and at her institution, a social worker sits in during the informed consent discussion, and contacts the patient by phone several days later to ensure that he or she still understands the nature and risks of the study and still wants to participate. Taking a cue from this, ►



▶ perhaps HTC social workers should review patients' knowledge and understanding of the study at regular intervals.

### AS THE STUDY PROCEEDS

What are some other important ways in which HTC social workers can assist patients and the research team during the course of a study? Many of them are simply the things that social workers already do as part of comprehensive care at HTCs.

During the course of a gene transfer study, the HTC social worker can check with the patient at study visits to assess his mental state, coping ability and resources and offer help as needed. Sometimes practical needs emerge, such as transportation to study-related follow-up appointments. If a social worker identifies more serious concerns, such as a change in a patient's mental status or uncertainty about wanting to continue to participate in the study, then he or she should notify the research physician immediately.

When a gene transfer study concludes, lab and clinical monitoring continue indefinitely to ensure patient safety. The social worker can follow up with participants to elicit reactions, impressions and feelings about having participated in and completed the study. Most patients who have been part of such novel, groundbreaking studies have impressions to share, both negative and positive. The social worker can address any lingering doubts or anxieties that patients may have, making referrals to psychiatric or other counseling specialists if warranted.

### CALMING THE HYPE

The tremendous potential of gene transfer makes this an exciting time for the hemophilia community; every newsletter contains a gene therapy update and every meeting agenda lists gene therapy as a topic for discussion or presentation. It is almost unavoidable that all of this attention boosts patients' fam-



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of a gene transfer  
study, the HTC  
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visits to assess his  
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resources.

ilies' and HTC professionals' expectations about gene transfer. HTC staffs can expect that many of their patients and families will have high hopes—and possibly unrealistic expectations—about gene transfer research, whether or not they are participating in studies or considering doing so. Despite their enthusiasm because of recent advances, HTC teams must be careful to accurately portray the current status of gene transfer research to patients and families. Social workers may play a crucial role because of their extensive training in communication and nonreactivity (staying rooted in facts even when others react emotionally to an issue). Social workers are often a primary point of contact for patients, and therefore must be able to field general questions about the status of gene transfer research in hemophilia.

A presentation given at NHF's 52nd Annual Meeting in Anaheim, California, in 2000 illustrated the importance of the language that is used when discussing gene transfer research. Eric Juengst, PhD, of Case Western Reserve University, called attention to the use of the term "gene therapy," which implies that the experimental gene transfer procedures will be "therapeutic" and will have clinical benefit. Dr. Juengst pointed out that the correct term is actually "gene transfer," which accurately describes the procedure without implying success or clinical improvement.

An article published previously in *HemAware* (*It's time for clarifying a cure*, Feb. 2001;34-35) written by Jan van Eys, PhD, MD, also urges caution in using the word "cure" in the hemophilia field lightly, as there are many components involved that may lead to a cure. It is important, therefore, to take care not to equate successful completion of a phase I gene transfer study with a "cure" for hemophilia. Even if a small number of people with hemophilia were to participate in gene transfer procedures, permanently increase their clotting factor activity, eliminate the risk of hemorrhage and ▶

► have no side effects of any kind, this could not be interpreted as a cure for hemophilia. According to Dr. van Eys, cure is comprised of physical, psychological and social aspects. A person with hemophilia whose clotting factor activity becomes normal because of a gene transfer intervention still bears the identity and experiences of his pre-intervention life as a person with hemophilia. As Dr. van Eys writes, "A chronic affliction leaves its scars." Dr. van Eys differentiates between private health versus public health interpretations of the word "cure." A disease cannot be considered truly "cured" until it has been eradicated from the human race. Although it is truly exciting to hear of people with hemophilia receiving gene transfers that result in higher clotting factor levels and decreased bleeding, it is sobering to imagine how long it will be, if ever, until gene transfer is safe, effective, affordable and available to every person with hemophilia in the world—until we can consider hemophilia to be truly "cured."

#### **OPPORTUNITIES FOR SOCIAL WORK RESEARCH**

Currently, gene transfer research exists mainly in the scientific realm; it is gradually being introduced to the clinical realm. Social workers are in a unique position to raise issues that have not been a focus of scientific and medical research. For example, how are the first hemophilia gene transfer patients coping? Are they generally satisfied with their participation and outcomes? Do they have regrets? What are they telling other people with hemophilia about their experiences with gene transfer studies? How can these findings be used to improve the gene transfer protocols yet to come? How are HTC teams communicating gene transfer results to their patient populations in general? Do patients have shared concerns or complaints that social workers must convey to physicians, nurses and researchers? NHF now offers a



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social work research grant that could support investigations into these crucial issues.

#### **CALL TO ACTION**

Hemophilia gene transfer research has been limited to very few sites thus far. Most likely, the social work role has developed differently at each site. Some social workers may be integral to the research team, and others may not interact with staff or patients about gene transfer studies at all. It would seem beneficial for these few social workers to have an informal network, perhaps via e-mail, to share their experiences and suggestions with each other. As gene transfer research becomes more widespread, more HTC social workers will need information and recommendations about interacting with staff and patients to bring about the best possible outcomes; an informal network could be very helpful. Perhaps social workers in other fields that involve gene transfer research (ie, cystic fibrosis) could also participate. I invite any HTC social workers who have worked with patients enrolled in gene transfer studies or are doing so now to contact me and share their thoughts about forming a network for the purpose of exchanging experiences and ideas at (412) 209 7286 or e-mail [dstandish@mail.com](mailto:dstandish@mail.com).

An HTC social worker has many skills to offer to both the research team and the patients. As this exciting research progresses, social workers will have the opportunity to learn from researchers, patients enrolled in studies and social work colleagues around the country. The social work contribution to gene transfer research is a mostly untapped resource that could be furthered by establishing an informal network. Ultimately, social workers can do their part to ensure that today's gene transfer study participants and tomorrow's beneficiaries of such research are well informed and well supported by their HTC care team. 📍