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The potential of treatments based on human stem cells is stimulating tremendous excitement and hope for HD families and researchers. Human stem cell lines derived from skin samples of HD patients or at risk individuals are particularly exciting.

These cells have many possible applications. They might be directed to become brain cells so the disease can be studied in a human context, or they might be used to efficiently screen drug candidates to accelerate the discovery of new treatments.

Stem cells also might be used to treat HD directly by “nursing” diseased brain cells or perhaps even by delivering new and healthy neurons to the brain.

These and other therapeutic strategies are at various stages of development and hold great promise. However, we remain cautious in our efforts to ensure that stem cell–based treatments are truly safe as well as effective. A recent trial in Russia, where nervous system tumors developed in a child given “neural stem cells”, highlights the critical importance of safety regulations. Exciting as they are, stem cell-based approaches require extensive study and evaluation in animal models of HD and caution before testing in human patients.

The information regarding this trial and guidelines from the International Society for Stem Cell Research is attached below.

Sincerely,

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