Stem Cell 101

What is a stem cell?
A stem cell is a parent cell in the body that has two specific abilities:

- It can reproduce, or make more of itself – this is called self-renewal.
- It can turn itself into all types of tissue in the body – this is called differentiation.

Where are stem cells found?
There are two types of stem cells: embryonic stem cells, found in embryos, and adult stem cells, which can be found in nearly every tissue or organ of the body.

Embryonic stem cells have the ability to become any type of cell, while most adult stem cell populations are partially specialized, and more restricted in which types of cells they can become. For example, stem cells found in muscle generally make muscle and stem cells found in the skin generally form skin.

However, some research suggests that certain adult stem cells have the ability to act more like embryonic stem cells. [Note: any stem cell that is not derived from an embryo is most often referred to as an adult stem cell, even if from the umbilical cord or cord blood of a newborn. In the circumstance where stem cells are derived from fetal tissues, some will refer to these as fetal stem cells.]

What are iPS cells?
iPS cells are embryonic-like stem cells created from adult tissues as a result of genetic modification. Several years ago, researchers found that the introduction of specific genes known to be turned on in the embryonic stem cells could cause non-stem cells from adult tissue to revert into stem cells with much of the adaptability and of embryonic stem cells. This discovery created excitement for obvious reasons: suddenly, researchers had the potential ability to create patient-specific stem cells that would be less likely to be rejected by the body if they were transplanted.

Where does umbilical cord blood fit in?
Just as in bone marrow, stem cells capable of developing blood cells are present in umbilical cord blood. At birth, these cells can be collected and preserved for use in transplants, with no harm to the mother or child. Umbilical cord blood has been successfully used to treat various bone marrow and blood diseases, including cancer, as well as a variety of genetic conditions. More than 20,000 umbilical cord blood transplants have been performed for treating diseases of the marrow/blood and immune system or repairing the marrow/blood and immune system after high doses of chemotherapy and radiation.

How were stem cells used in the EB clinical trial?
Stem cells were used for two reasons:

1) to see if stem cells would bind to the damaged skin and repair it, and;

2) to replace the patients own marrow that had been destroyed by high doses of chemotherapy.

Because the stem cells were from a healthy tissue sample collected from matched or partially-matched (sometimes related) donors, high doses of chemotherapy had to be given to destroy the patient's immune system so that they could not reject the new transplanted stem cells. These transplanted stem cells were needed to replace the cell's recognized by the recipient's immune system, which from then onward would be that of the donor. If successful, the blood, marrow and immune system would forever be the donor's, no longer capable of rejecting donor stem cells. On the basis of substantial preclinical work, we hypothesized that a rare subpopulation of stem cells residing in the marrow has the capacity to home to the skin and repair it.

How did it work?
Much more research needs to be done to exactly understand how the treatment actually worked. First, researchers know that all patients evaluated have had some improvement--some more than others. Second, it usually takes months to see a response to the treatment, although some responses occurred quite rapidly. Third, physicians have observed improved resistance to blistering over time, indicating that the skin has functionally improved. Fourth, this improvement is not solely due to an increase in collagen 7 or normal anchoring fibrils, which are responsible for bonding the skin to the body. Fifth, a substantial proportion of the skin is made up of donor cells, with some but not all being inflammatory cells. And lastly, the response lasts long-term with a systemic impact.

Do we need to do research on both types of stem cells?
At this time, yes. Scientists do not yet know which types of stem cells will be best in treating various diseases. Currently, embryonic stem cells are still the "gold standard" due to their ability to most easily become any types of cells in the body. Continuing to study both adult and embryonic stem cells allows scientists to compare outcomes.

Where can I go for more scientific information about stem cells?
The National Institutes of Health's Web site has extensive information about stem cells: http://stemcells.nih.gov/info/basics/