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Key Action Items for the Stem Cell Field: Looking Ahead to 2014

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ABSTRACT

The stem cell field is at a critical juncture in late 2013. We find ourselves buoyed by building momentum for both transformative basic science discoveries and clinical translation of stem cells. Cellular reprogramming has given the field exciting new avenues as well. The overall prospect of novel stem cell–based therapies becoming a reality for patients in the coming years has never seemed higher. At the same time, we face serious challenges. Some of these challenges, such as stem cell tourism, are familiar to us, although even those are evolving in ways that require adaptability and action by the stem cell field. Other new challenges are also emerging, including an urgent need for formal physician training in stem cells, regulatory compliance balanced with innovation and U.S. Food and Drug Administration reform, and savvy educational outreach. Looking ahead to 2014, both the challenges and opportunities for the stem cell field require a proactive, thoughtful approach to maximize the potential for a positive impact from stem cell advances. In this study, I discuss the key action items for the field as we look ahead to the coming year and beyond.

The stem cell field needs to take action and responsibility for training a new generation of physicians who are excited to be stem cell specialists.

Training a new generation of stem cell clinicians

Patients are seeking stem cell interventions for a wide range of illnesses and injuries. In turn, a growing number of physicians are working to meet this demand. Most doctors in the United States and globally have received effectively no training at all in the basic science or clinical translation of stem cells. Even the growing subset of doctors specifically interested in performing stem cell–related medicine have little if any formal training in stem cells. The increasing demand for stem cell training is leading to dubious crash courses usually offered over a 2-day period. The argument that "some training is better than none" does not apply in this case. In fact, such short courses enable doctors to take disproportionate risks by giving them the false confidence to perform stem cell interventions that they are not actually adequately trained to provide. In this way, the training gap in the stem cell field puts both the patients as well as the doctors and their practices at great risk.

We need a better training solution. I recently called for the development of formal academic stem cell training programs for physicians in the form of a 1-year fellowship or subspeciality training in stem cell–based cellular and regenerative medicine [1]. To my knowledge, no such formal programs currently exist in the United States. The dubious training courses will continue to fill the gap as long as it persists, dangerously enabling untrained doctors to be stem cell providers for a growing number of patients. More broadly, a training program of 2 days is not enough for a physician to become qualified to act as a specialist in any area of medicine. It is a mistake to believe that stem cell transplants require any less training. The stem cell field needs to take action and

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responsibility for training a new generation of physicians who are understandably excited to be stem cell specialists and help patients in this way.

Balancing act: Both working with the FDA and advocating for responsible reforms at the agency

The U.S. Food and Drug Administration (FDA) is performing critical regulatory oversight of stem cell–related medical procedures, even with a nearly static budget over 5 years [101]. At the same time, change is needed at the FDA and other regulatory bodies around the globe in terms of how innovative stem cell therapies are evaluated and approved. A key action item in 2014 consists of both supporting current appropriate FDA regulation of stem cell therapies and helping the agency make important course corrections for regulation of stem cell–based therapies in the future. Dubious stem cell interventions are increasing in number, putting a growing number of patients at risk. Unfortunately, at the same time, the conventional regulatory pipeline has an unacceptably slow transit time. In 2014, we should continue to support the FDA but actively work toward specific FDA reforms to accelerate the approval of compliant, innovative stem cell treatments. Changes such as careful expansion of compassionate use of stem cells for patients with terminal diseases such as amyotrophic lateral sclerosis, also known as Lou Gehrig's disease, are needed for the FDA of tomorrow. However, to avoid unintended consequences and manipulations as occurred in Italy in 2013 [2], the widened scope of compassionate use in the United States must be strictly governed by clear rules such as limiting participation to patients with terminal illnesses.

Another change I am advocating for at the FDA in the coming year is more openness and accessibility. The more effective FDA of the future will have regulations that are readily understandable by an educated lay public as well as scientists and physicians. In contrast, even so-called experts today often find the language used by the FDA challenging to decipher.

I also believe that we must help the FDA adopt a more proactive approach to regulation, whereby it identifies providers of stem cell interventions and rapidly intervenes in cases where issues of compliance and patient safety are evident. In the current paradigm, where the absence of complaints can see a few years go by before the FDA audits the stem cell clinics, patient risk is unnecessarily high. This situation also presents risks for the stem cell field as a whole; even a few serious adverse events by noncompliant stem cell providers can tarnish the whole field.

Stem cell tourism hits home: Global and local action needed

The stem cell field has been confronting stem cell tourism—where patients travel from home usually to a region of lower regulatory oversight to receive stem cell–based interventions—for many years [3,4]. However, in the last 2 years, stem cell tourism has been rapidly morphing into a truly global phenomenon. In the new reality, noncompliant stem cell interventions are now offered so ubiquitously that a significant number of patients are no longer traveling long distances because it is simply no longer necessary. For example, in the U.S. stem cell clinics, physicians offering stem cell interventions as add-ons to their regular practices have proliferated quickly in 2012–2013 across the country.

We may soon find a new reality in 2014, if we have not already reached that point, in which more Americans receive noncompliant stem cell therapies inside the United States than outside its borders. By no means does this imply that stem cell tourism in the traditional sense of the term—involving extensive travel—is dead. To be clear, very large numbers of people globally and in the United States still travel to other countries for stem cell interventions, but local noncompliant options have greatly expanded. This is also reflected in increased advertising of domestic stem cell interventions. As the field looks to the year ahead, these trends are predicted to continue and pose new challenges. Increased patient access to noncompliant stem cell interventions is a recipe for growing negative outcomes. As mentioned earlier in the previous section, the FDA should take a proactive approach to dubious stem cell operations, but societies such as the International Society for Stem Cell Research (ISSCR) must also be more actively involved than they are today. Whereas in the past, ISSCR had been a more active participant in promoting stem cell compliance, the effort was substantially curtailed in 2011 at the reported threat of litigation from specific dubious stem cell clinics [5]. Nonetheless, ISSCR and other societies can have a more active role by highlighting the progress of compliant businesses in the stem cell field (for example, inviting compliant stem cell business leaders to speak at the annual ISSCR meeting in prominent positions), lobbying the FDA to take more proactive roles, and providing guidance on regulatory matters. The current system whereby the burden is placed on individual concerned citizens—whether stem cell scientists, ethicists, or others—to raise concerns with the FDA about stem cell clinics that are noncompliant and put patients at risk, is simply untenable and forces these concerned individuals to take their own personal risks.

Savvy stem cell educational outreach in the social media era

Another trend in the stem cell field is the growing presence of stem cells in pop culture and social media. The fact that stem cells have permeated the consciousness of everyday people around the world provides a greater opportunity to educate through outreach and to sustain or even grow appropriate funding levels for critical research. At the same time, the ubiquitous presence of stem cells in movies, TV

shows, magazines, and most strikingly on the Internet presents new challenges as well. For instance, stem cell myths and misinformation are often passed along to consumers using pop culture avenues. In the movies and other pop culture productions, stem cell technology is frequently invoked to perform almost magical medical or biological outcomes [102], perhaps leading to greatly exaggerated expectations of the public about what stem cells can achieve.

The vast majority of people interested in stem cells around the world do not learn about stem cells from scientific journals. Instead, they receive information and unfortunately misinformation from the Internet. Stem cell social media is an increasingly powerful conduit for education outreach, but is also co-opted by purveyors of noncompliant stem cell interventions to attract customers. If we do not actively use social media, it will continue to be dominated by those simply interested in making money off of stem cells. For example, despite some progress over the last few years, the number of positive advocacy and education-oriented stem cell blogs remains largely the same as when I started blogging in 2010 [6]. In 2014, a critical action item for the stem cell field and leading organizations such as ISSCR is to embrace the power of social media as a tool for outreach and education. In the coming year, ISSCR should begin its own blog for advocacy and education. An excellent example to follow is the Signals Blog by The Stem Cell Network [103].

We should all be excited and encouraged by the increasing prospect of compliant, evidence-based stem cell treatments approaching on a nearer horizon. However, there is a strong possibility of what I term "the dilution effect" in the near future, whereas in the popular consciousness the term "stem cell treatment" becomes equated with noncompliant interventions. In fact, this dilution effect may already be occurring. People around the world are increasingly exposed to advertising of one kind or another or word of mouth for dubious stem cell therapies. One danger with dilution is that the public lowers their opinion of the rigorousness and ethics of stem cell–based medicine as a field. Paradoxically, at the same time, the public may increasingly possess unrealistic expectations of stem cells such as imminent, outright cures. The stem cell community needs to take action in the coming year to use social media to promote evidence-based stem cell medicine and distinguish it from noncompliant options.

Looking to 2014 and beyond: Breaking down barriers and building bridges

Even within the legitimate, compliant stem cell community, there are too many divisions. As we look ahead to the coming year, there is no room for stem cell silos and walls that in effect weaken the field. For too long the stem cell world has largely been divided into different camps: scientists, physicians, patients, advocates, politicians, and others. We need more of a cooperative effort in which these groups work together to advocate for accountability and positives changes. Efforts such as the World Stem Cell Summit that are inclusive have been a major success and made substantial progress in building bridges. Other efforts such as bringing different parties together in the stem cell field by using social media have also been helpful. A more open, interactive FDA needs to be part of this team as well. The future seems bright for 2014 and beyond for the stem cell field. I am very optimistic about the stem cell field transforming medicine, for example, but wisdom dictates that if we take wise actions now for course corrections, we can substantially boost the odds of even greater progress.

Key Trends in the Stem Cell Field to Watch for in 2014

- Social media and pop culture influence
- Changes in formal physician training
- Regulatory compliance balanced with innovation
- Changes and possible receding of "stem cell tourism" rates
- A more cooperative environment between scientists, physicians, patients, advocates, politicians, and others.

Author disclosure statement

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REFERENCES

Primary Literature

1. Knoepfler PS. (2013). Call for fellowship programs in stem cell-based regenerative and cellular medicine: new stem cell training is essential for physicians. Regen Med 8:223–225.
2. Abbott A. (2013). Stem-cell ruling riles researchers. Nature 495:418–419.
3. Hyun I. (2013). Therapeutic hope, spiritual distress, and the problem of stem cell tourism. Cell Stem Cell 12:505–507.
4. Mason C and E Manzotti. (2010). Defeating stem cell tourism. Foreword. Regen Med 5:681–686.
5. Ledford H. (2011). Stem-cell scientists grapple with clinics. Nature 474:550.
6. Knoepfler P. (2011). My year as a stem-cell blogger. Nature 475:425.

Websites

101. Knoepfler P. As stem cell industry explodes can a static FDA keep up? Knoepfler Stem Cell Blog. September 19, 2012. www.ipscell.com/2012/09/as-stem-cell-industry-explodes-can-a-static-fda-keep-up/
102. Knoepfler P. Emma Stone dishes on Spiderman and stem cells: very cool. Knoepfler Stem Cell Blog. June 27, 2012. www.ipscell.com/2012/06/emma-stone-dishes-on-spiderman-and-stem-cells-very-cool/
103. Signals Blog: Stem Cell Network Blog. www.signalsblog.ca/