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June 24, 2020

The Honorable Stephen Hahn
Commissioner
U.S. Food and Drug Administration
10903 New Hampshire Ave
Silver Spring, MD 20993-0002

Dear Commissioner Hahn,

On behalf of the International Society for Stem Cell Research (ISSCR), I write to thank you for the FDA's recent enforcement activities against businesses marketing unproven stem cell-based interventions. The ISSCR is the leading professional organization of stem cell researchers and represents more than 4,000 members in the US and around the world. Our members are scientists, clinicians, ethicists, and educators dedicated to the responsible advancement of stem cell research and its translation to the clinic. We support the FDA's crucial role in protecting the public health by ensuring that new therapies are proven safe and effective before being sold to patients.

Businesses prematurely marketing unproven stem cell-based interventions are exploiting the COVID-19 pandemic and the absence of proven treatments to market unproven "regenerative therapies" and other stem cell-based interventions claiming to boost immune system function, prevent SARS-CoV-2 infection, or mitigate the effects of infection on tissue function. These businesses often cite unconnected and anecdotal findings as evidence that their products are safe and effective. They sell scientifically implausible products that are unlikely to provide any benefit and may pose serious risks to patients. Many of the cell-based interventions provided by unscrupulous business are non-homologous uses of the cells and require FDA approval. Furthermore, all cell therapy products come with processing and contamination risks that should be assessed by regulators. The ISSCR applauds the FDA's warnings against the premature use of unproven stem cell treatments and the recent untitled and warning letters against businesses illicitly marketing stem cell-based interventions. We urge the FDA to continue boosting

Promoting excellence in stem cell science and applications to human health.

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enforcement activities with particular attention to businesses seeking to exploit the fear of COVID-19 to market unproven therapies.

The FDA's regulatory oversight mission is also critical to protecting patients as it reviews IND's for COVID-19 trials. The pandemic has made that mission more challenging with political and public pressure on the agency to expeditiously review INDs and approve new treatments. However, the FDA must continue to insist on robust and sound pre-clinical safety and efficacy data before allowing first-in-human clinical trials for experimental therapies for COVID-19. There have been many public claims from companies related to proposed cell therapies for COVID-19 that lack any credible basis in science. We know from experience with other products in this area that companies who are willing to sell therapies without regard to whether they are scientifically plausible are also often willing to ignore good manufacturing practices: there have now been many cases in which these products have been found to be contaminated by pathogens. Consequently, these products should only proceed to first-in-human clinical trials after developers have minimized safety risks by rigorously characterizing and assaying the toxicity and tumorigenicity of each product and showing they follow good manufacturing practices. These products should be required to have a plausible mechanism of action and plausible patient benefit before being tested in clinical trials.

The premature development of products that lack a plausible scientific basis puts patients at risk and wastes resources that could be used to develop more rational therapies. Such products also threaten the public health because Right to Try laws enable developers to evade important aspects of FDA regulation and to prematurely sell products to patients before they are proven safe and effective. Right to Try has increased the stakes for early phase clinical trials and elevated the need for rigorous assessment of preclinical data.

Thank you for considering our views on the FDA's enforcement activities and our recommendations regarding the development of new therapies. If the ISSCR can clarify any of these views or be of assistance, please contact Eric Anthony, ISSCR's Director of Policy at eanthony@isscr.org.

Sincerely,



Deepak Srivastava, MD
President, ISSCR
President, Gladstone Institutes