Scientific Research Publishing

Open Journal of Regenerative Medicine, 2022, 11, 55-62
https://www.scirp.org/journal/ojrm
ISSN Online: 2169-2521
ISSN Print: 2169-2513

Huu S. TIEU Request for FDA to Establish Regenerating Human Cells as Law on December 13, 2016 President Barack H. Obama Signed the 21st Century CURES Act into FDA Regulation and Law

Huu S. Tieu, Martin F. Loeffler, Edgar A. Ayala

Golden Sunrise Nutraceutical, Inc., Porterville, USA
Email: htieu@goldensunrisenutraceutical.com

How to cite this paper: Tieu, H.S., Loeffler, M.F. and Ayala, E.A. (2022) Huu S. TIEU Request for FDA to Establish Regenerating Human Cells as Law on December 13, 2016 President Barack H. Obama Signed the 21st Century CURES Act into FDA Regulation and Law. *Open Journal of Regenerative Medicine*, **11**, 55-62.
https://doi.org/10.4236/ojrm.2022.113004

Received: August 25, 2022
Accepted: September 27, 2022
Published: September 30, 2022

Abstract

Background and Aims: On November 24, 2009, Huu S. TIEU and Golden Sunrise Pharmaceutical, Inc. (Golden Sunrise) applied for the Technology and Innovation to be reviewed and evaluated by the U.S. Food and Drug Administration (FDA). In the review and evaluation, it was requested by Golden Sunrise designated the new indications for this application under Serious or Life-threatening conditions or diseases. Discussions followed with the FDA, Huu S. TIEU, and Golden Sunrise for FDA approval on new products and new indications on existing new Medical Technology and Innovation. It was agreed in Year-2015 that the FDA would take the request for new indications to the United States Congress to establish into FDA regulation and law. At that time the following was the FDA Guidance—"Emergency Use of a Test Article" is *exempt* from prior Institutional Review Board or Advisory Committee evaluation and approval, provided that such emergency use is reported to the Institutional Review Board within five working days after use. Expedited Institutional Review Board or Advisory Committee approval is not permitted in emergency use. There has been no funding to the authors for the writing or publication of this article. **Methods:** It was requested by Huu S. TIEU and Golden Sunrise in documents given to the FDA to have Serious or Life-threatening conditions or diseases indication be recognized by law. On August 08, 2015, the FDA responding to this request took the documentation produced by Golden Sunrise to the United States Congress on behalf of Golden Sunrise and Huu S. TIEU. This article encompasses the FDA regulatory

method as well as the discussion and results of the establishment of the FDA and the 21st Century Cures Act. **Results:** On December 13, 2016, H.R.34—114th United States Congress (2015-2016) 21st Century CURES Act was signed into law by President Barack H. Obama which included the Serious or Life-threatening indication to be written into the CURES Act. In summary, the 21st Century Cures Act is a landmark piece of legislation that enjoyed broad bipartisan support in United States Congress. The main goals of the Act are impactful and should transform future cancer, neurologic, and precision medicine or drug research as well as aid individuals with mental health is intended to facilitate the prompt approval of new agents and devices, clinicians should be aware of the types of data behind an approval and take this into consideration when developing illnesses and opioid dependence. However, some of the wording within the CURES Act regarding the drug and device approval process may bring pause to health care providers including pharmacists. Although this wording and implementing care plans and counseling patients. The 21st Century Cures Act was incorporated into laws and regulations by the FDA under § 3072 of the Act grants the Commissioner of Food and Drugs the authority to appoint and set the annual rate of pay for outstanding and qualified candidates to scientific, technical, or professional positions that support the development, review, and regulation of medical products.

Keywords

The 21st Century Cures Act, Therapeutic Protein, Serious or Life-Threatening Illness, ImunStem, Biologics Product, Therapeutic Biological Product

1. Introduction

The use of Golden Sunrise's new medical Technology and Innovation in application first began as an emergency use designation under the FDA Guidance for the Industry "Emergency Use of a Test Article" under FDA regulations Title 21 C.F.R. § 56.104(c) [1]; allows, the use of a test article on a human subject in a life-threatening situation in which no standard acceptable treatment is available, and which there is not sufficient time to obtain Institutional Review Board approval [2]. The new medical Technology and Innovation were used with the emergency standard in mind. When in a licensed doctor's care, the medical professional could invoke the "Emergency Use of a Test Article" criteria which allow a five day treatment with the Institutional Review Board or Advisory Committee involvement [3]. The physician/doctor standard was a successful way of using unapproved products when patients were in a life-threatening situation. This standard did not have a Serious or Life-threatening condition or disease yet established as an indication [4]. Huu S. TIEU and Golden Sunrise seeing the benefit to the patient with serious illness then requested that the FDA petition the United States Congress to pass legislation that would designate the Serious or Life-threatening conditions or diseases as an indication that doctors could

use for their patients without the continuous need for the Institutional Review Board or Advisory Committee oversight and a restriction of five days for treatment [5].

Huu S. TIEU and Golden Sunrise requested consideration for a new indication of Serious or Life-threatening conditions or disease and for new legislation to the FDA on November 24, 2009 through August 08, 2015 [6].

2. FDA Regulatory Method

Emergency use is not considered "Research" under the Department of Health and Human Services regulation and such emergency use is not considered "Research" as covered under Title 45 C.F.R. Part 46—Protection of Human Subjects [7]. The law was set down by the FDA for "Emergency Use of a Test Article" to be administered on a human subject in a life-threatening situation in which no standard acceptable treatment is available, and in which there is not sufficient time to obtain an Institutional Review Board or Advisory Committee approval or acceptance. This may provide benefits to a patient in a life-threatening situation for that time but it becomes a problem for the sponsor/manufacturer to carry out on a case-by-case basis and for the doctor or medical professional to provide proper treatment for patients in Serious or Life-threatening conditions or diseases. It is also required that consent be given by the patient though, many patients are not coherent in a life-threatening situation and family members may be apprehensive to allow other treatments. Huu S. TIEU seeing that the patient was not being fairly allowed new therapeutic treatments available to them requested the FDA petition the United States Congress to make a law and regulation that would allow Serious or Life-threatening conditions or diseases to become an indication so that the doctor could treat a patient more readily and provide the patient all treatments available to benefit a serious illness situation. The CURES Act also addresses the opioid crisis and allows for the improvement of mental health services [8].

3. FDA Established 21st Century Cures Act Discussion and Results

In Year-2015 the FDA requested that United States Congress create laws and regulations concerning issues that had come to light of the flaws and needs that existed concerning new medical Technology and Innovation or new drug products and their need to make it to the market faster than the existing FDA pathway methods at the time of the request. In this request, the FDA included the category and indication of Serious or Life-threatening conditions or diseases. This is part of what is now known as the 21st Century CURES Act and was requested by Huu S. TIEU and Golden Sunrise Pharmaceutical from August 20, 2008, through August 08, 2015. In the request, Huu S. TIEU asked the FDA for Serious or Life-threatening conditions or diseases to be considered as an indication. The United States Congress established the CURES Act as an Expedited

Review Prioritized [9]; Fast Track designation [10], Accelerate Approval designation [11], Breakthrough Therapy designation [12], and Priority Review program [13] pathway that would have approval of an "Application to Market a New or Abbreviation New Drug or Biologic for Human Use" in a short period of time (as short as sixty-days) if the drug was shown to be safe and effective, the CURES Act allows sponsors/manufactures to provide "Real-World Evidence" [14] [15], "Medical Billing" [16], "Data Summaries," [17], and "Predict Clinical Benefit" [18]. The United States Congress finished and established the new law called the 21st Century Cures Act and it was signed by President Barrack H. Obama on December 13, 2016 [19]. On January 02, 2017, the FDA received five hundred million (US$500000000.00) dollars to implement the program and established the FDA Regenerative Medicine Advanced Therapy division [20] to speed the evaluation and review of cell therapies, regenerative medicine, biologics products, or any combination product using such therapies or products if it "is intended to treat, modify, reverse, or cure Serious or Life-threatening conditions or diseases.

Accelerated Approval; Traditional approval requires that clinical benefit be shown before approval can be granted. Accelerated approval is given to some new drugs for Serious and Life-threatening illnesses that lack satisfactory treatments. This allows a New Drug Application (NDA) to be approved before measures of effectiveness that would usually be required for approval are available [21].

Huu S. TIEU and Golden Sunrise Pharmaceutical, Inc. requested the United States FDA for an indication for serious illnesses. The FDA requested the United States Congress to create laws and regulations for a new medical indication of Serious or Life-threatening conditions or diseases (**Figure 1**).

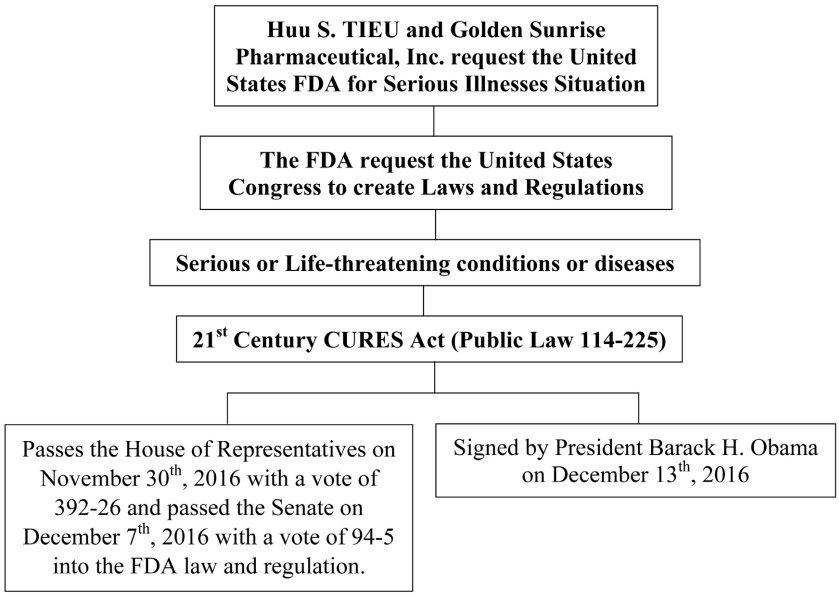

Figure 1. Request for new medical indication and progress to FDA laws and regulations.

The implementation of this set of laws and regulations was believed to benefit public health for the almost immediate access to new medical Technology and Innovation. New drug products that would not be available otherwise for the treatment of therapeutic proteins [22] or the therapeutic biologics [23] products treatment of patients would not be allowed without access to needed alternatives for drugs and therapies that may improve the outcomes of patients that have exhausted other human prescription drug products or therapies. The only alternative over time would be DEATH. Patients with serious illnesses, manufacturers, hospitals, and doctors would also benefit from this set of laws and regulations as in the previous designation for use of a drug product in a Serious or Life-threatening situation the manufacturer could only provide one sample "Emergency Use of a Test Article" patient for five days which is difficult and prohibitive to distribute and monitor as it makes the manufacturer to only produce one drug sample under Title 21 C.F.R. Part 56.104(c) and for the physician/doctor to only use the drug product up to five days. The 21st Century CURES Act establishes that a Serious or Life-threatening condition or disease is considered an indication and may be treated then billed to the United States Center for Medicare & Medicaid Services and other private insurance [24]. The FDA did not benefit from this new set of laws and regulations because under the Serious or Life-threatening conditions or diseases designation, the pathway of approval through the clinical trials was not required as it is considered unethical to use a randomized double blind placebo studies where over half of the test group could die from lack of proper treatment, as would be the case in a Serious or Life-threatening situation for which there is established effective therapy [25].

On February 16, 2017, Huu S. TIEU and Golden Sunrise requested the FDA Regenerative Medicine Advanced Therapy for a Breakthrough Therapy designation, this designation would treat patients with Serious or Life-threatening conditions or diseases from the Regenerative Medicine Advanced Therapy division, Golden Sunrise had an FDA New Drug Application No.: 204701 and National Drug Code Directory No. 70642-001-01 [26]. 1) During this process discussions took place between Huu S. TIEU, Golden Sunrise, and the Regenerative Medicine Advanced Therapy division. On March 25, 2019, Golden Sunrise received the FDA Inspection Report and Establishment Identifier No.: 3012327979 [27]. After the Regenerative Medicine Advanced Therapy division designation was applied to ImunStem product, the Golden Sunrise documents were then moved forward to the Product Jurisdiction Officer on August 27, 2019. After all documents were reviewed and accepted, the Golden Sunrise product was classified as FDA-Regulated Medical Product and met the requirements for acceptance under the FDA Significant Scientific Agreement standards. 2) During the Coronavirus pandemic in January 2020 the FDA created the FDA Coronavirus Treatment Acceleration Program division on March 30, 2020, to address the COVID-19 pandemic. Huu S. TIEU and Golden Sunrise applied and submitted a cover letter for the FDA Coronavirus Treatment Acceleration Program division with the indicated treatment for COVID-19 and Serious or Life-threatening conditions or

diseases on April 03, 2020. On September 15, 2020, after submitting patient medical report results for COVID-19 under the Priority Review Expedited No.: 2020-2867 CR2020-2596 to the FDA Industry for Biologics department. The FDA Coronavirus Treatment Acceleration Program division moved the Golden Sunrise documents forward to the FDA Center for Drug Evaluation and Research department and FDA Center for Biologics Evaluation and Research department for review and evaluation. After the FDA Center for Drug Evaluation and Research department and FDA Center for Biologic Evaluation and Research department reviewed and evaluated the patient medical report results (Real-World Evidence) the submission moved forward to the FDA Product Jurisdiction Officer department and it was designated Golden Sunrise products and Plans of Care as FDA-Regulated Medical Products, which was established in the FDA Pharmacy department [28]. On November 13, 2020, United States Center for Medicare & Medicaid Services and other private insurance agreed with value payment to Golden Sunrise treatment under procedure code—Cellular Therapy M0075.

4. Conclusion

Huu S. TIEU submitted a cover letter on November 24, 2009, which requested the FDA to review and evaluate Golden Sunrise Pharmaceutical products for a new indication of Serious or Life-threatening conditions or diseases without randomized double blind placebo studies. After the United States Congress created the 21st Century CURES Act (Public Law 114-255) "Passed the House of Representatives on November 30th, 2016 with a vote of 392-26 and passed the Senate on December 7th, 2016 with a vote of 94-5 signed into law by President Barack H. Obama on December 13th, 2016". This law covers Serious or life-threatening conditions or diseases as a new indication.

Acknowledgements

The authors would like to take this time to thank President Barack H. Obama for the signing of the 21st Century Cures Act into FDA Law and Regulation. The authors also like to thank the United States Congress for their diligent efforts to gather the content and in the creation of this Law and Regulation to provide improvement to the care of patients in medical need.

Conflicts of Interest

The authors declare no conflicts of interest regarding the publication of this paper.

References

[1] Title 21 C.F.R. § 56.104(c). https://www.ecfr.gov/current/title-21/chapter-I/subchapter-A/part-56/subpart-A/section-56.104

[2] Emergency Use a Test Article under Title 21 C.F.R. Part 56.102(d). https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfcfr/cfrsearch.cfm?fr=56.102

[3] Investigation New Drug Application under Title 21 C.F.R. § 312.310 Individual Patients, Including for Emergency Use and under Title 21 C.F.R. § 312.310(a) Criteria. https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfcfr/CFRSearch.cfm?fr=312.310

[4] Title 21 C.F.R. § 312.305 Requirements for All Expanded Access Uses. https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfcfr/CFRSearch.cfm?fr=312.305

[5] 21st Century CURES Act, H.R. 34-114th Congress (2015-2016). https://www.congress.gov/bill/114th-congress/house-bill/34

[6] Tieu, H. and Loeffler, M. (2022) FDA Approves First Dietary Supplement under New Drug Application with the Indication of Serious or Life-Threatening Illnesses. *Pharmacology & Pharmacy*, **13**, 529-544. https://doi.org/10.4236/pp.2022.1312038

[7] U.S. Code of Federal Regulation Title 45 C.F.R. Part 46—Protection of Human Subjects (1974). https://biotech.law.lsu.edu/research/fed/45cfr46.htm

[8] Cole, D.M., *et al.* (2018) The 21st Century Cures Act Implementations for the Reduction of Racial Health Disparities in the U.S. Criminal Justice System: A Public Health Approach. *Journal of Racial and Ethnic Health Disparities*, **5**, 885-893. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5943192 https://doi.org/10.1007/s40615-017-0435-0

[9] Expedited Programs for Regenerative Medicine Therapies for Serious Conditions; Guidance for Industry (2019, February 19). https://www.federalregister.gov/documents/2019/02/19/2019-02691/expedited-programs-for-regenerative-medicine-therapies-for-serious-conditions-guidance-for-industry

[10] FDA Fast Track (2018). https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track

[11] FDA Accelerate Approval (2018). https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/accelerated-approval

[12] FDA Breakthrough Therapy (2018). https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/breakthrough-therapy

[13] FDA Priority Review (2018). https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/priority-review

[14] Real World Evidence—Wikipedia. https://en.wikipedia.org/wiki/Real_world_evidence

[15] Gabay, M. (2017) 21st Century Cures Act. *Hospital Pharmacy*, **52**, 264-265. https://doi.org/10.1310/hpx5204-264 https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5424829

[16] HCPCS Code Details—M0075. https://hcpcs.codes/m-codes/M0075

[17] Black, J.R., *et al.* (2018) Health Information Blocking: Responses under the 21st Century Cures Act. *Public Health Reports*, **133**, 610-613. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6134556 https://doi.org/10.1177/0033354918791544

[18] 21st Century Cures Act (2016, December 13). https://www.cureffi.org/2016/12/13/21st-century-cures

[19] The 21st Century CURES Act—Wikipedia. https://en.wikipedia.org/wiki/21st_Century_Cures_Act

[20] 500 Million Dollars over 9 Years: FDA Details Plan for New "Cures" Funds. https://www.raps.org/regulatory-focus%E2%84%A2/news-articles/2017/7/$500m-over-9-years-fda-details-plan-for-new-cures-funds

[21] FDA's Drug Review Process: Continued (08/24/2015). https://www.fda.gov/drugs/information-consumers-and-patients-drugs/fdas-drug-review-process-continued

[22] Biopharmaceutical—Wikipedia. https://en.wikipedia.org/wiki/Biopharmaceutical

[23] Frequently Asked Questions about Therapeutic Biological Products (07/07/2015). https://www.fda.gov/drugs/therapeutic-biologics-applications-bla/frequently-asked-questions-about-therapeutic-biological-products

[24] Centers for Medicare & Medicaid Services—Wikipedia. https://en.wikipedia.org/wiki/Centers_for_Medicare_%26_Medicaid_Services

[25] Who Wins and Loses with the 21st Century Cures Act? https://www.pbs.org/newshour/health/wins-loses-21st-century-cures-act

[26] 70642-001-01 NDC Code. ImunStem. Human Prescription Drug November 03, 2018. https://www.hipaaspace.com/medical_billing/coding/national.drug.codes/70642-001-01

[27] Drug Establishments Current Registration Site—FDA. https://www.accessdata.fda.gov/scripts/cder/drls/default.cfm

[28] Tieu, H. and Loeffler, M. (2022, October 19) Life Is Quantum Biology Effects Explained from the Schrödinger Equation, Serious or Life-Threatening Conditions or Diseases, and COVID-19 Results. *Journal of Biosciences and Medicines*, **10**, 164-172. https://doi.org/10.4236/jbm.2022.1010013