January 19, 2017

Mario Morais
4 Glen Road, Apartment # 103
Hudson, MA, 01749-1365

Re: Appeal from FDA-2012-P-0119 and FDA-2013-P-0735

Dear Mr. Morais:

This letter responds to your appeal dated July 4, 2014,[1] and received by the Food and Drug Administration (FDA or the Agency) on July 15, 2014. Your appeal states that the Agency's June 19, 2014, response addressed your petition FDA-2013-P-0735 (dated June 1, 2013) but did not respond to your petition FDA-2012-P-0119 (dated December 5, 2011). In your appeal you request that FDA respond to petition FDA-2012-P-0119 and grant several other requests.

We note that you cite 21 CFR 12.125(a) as the authority for your appeal of the Agency's response to your citizen petitions. This section of FDA's regulations sets out the rules for formal evidentiary hearings and does not apply to citizen petitions. We note that you did not submit your appeal to the Agency under the procedures laid out in 21 CFR 10.33 regarding administrative reconsideration of action, and therefore we do not consider the letter to be an official petition for reconsideration. We have, however, considered the information submitted in your Petition, our response to the Petition, and your July 2014 submission.

For the reasons described below, your request is granted in part and denied in part.

I. BACKGROUND

Your petition FDA-2012-P-0119 (dated December 5, 2011) requested that FDA: (1) issue a statement that checking the blood drainage from the brain and "fixing it" (using the "liberation procedure") may benefit patients with neurological problems; (2) create an "unapproved drug category that requires minimum FDA forms maybe one FDA form"; (3) permit all interested patients to participate in clinical trials and let all participants receive the drugs being tested; (4) make "the drug BB:7075 as an AIDS treatment" available to U.S. citizens and ask the National Institutes of Health (NIH) to test it; (5) permit patients to have more influence in drug approval decision-making; and (6) provide safety information from investigational new drug trials. The petition contained a variety of information from published articles and other sources in support of your requests. The Agency provided you a response dated August 1, 2012, stating that it had not yet resolved the issues in your petition due to the need to address other Agency priorities and that the petition would be responded to as soon as possible "given the numerous demands on the Agency's resources."

[1] We note that the first page of your appeal letter is incorrectly dated July 04, 2012. In this response we will refer to the correct date of July 04, 2014, which appears on the second page of your letter.

Subsequently you filed your petition FDA-2013-P-0735 (dated June 1, 2013). This petition requested that FDA: (1) send your comment on the CCSVI (Chronic Cerebrospinal Venous Insufficiency) procedure (also known as "liberation procedure") to the Executive Secretariat; (2) include your comment on the CCSVI procedure in the FDA folder with the Agency's news release warning about this procedure; and (3) answer your positive comment about the CCSVI procedure by phone. FDA responded to this petition in its letter dated June 19, 2014.

The Commissioner may grant a petition for reconsideration if the Commissioner determines the petition to be in the public interest and in the interest of justice (21 CFR 10.33(d)). Section 10.33(d) provides that the Commissioner shall grant a petition for reconsideration if the Commissioner determines that all of the following apply:

(1) The petition demonstrates that relevant information or views contained in the administrative record were not previously or not adequately considered.
(2) The petitioner's position is not frivolous and is being pursued in good faith.
(3) The petitioner has demonstrated sound public policy grounds supporting reconsideration.
(4) Reconsideration is not outweighed by public health or other public interests.

A petition for reconsideration may not be based on information and views not contained in the administrative record on which the decision was made (21 CFR 10.33(e)).

Your request asks FDA to grant 4 items as follows: (1) Include your comment in the FDA folder for the procedure CCSVI; (2) provide a formal response to your comment on CCSVI by telephone from someone in the Center for Devices and Radiological Health (CDRH); (3) address and answer your first citizen petition FDA-2012-P-0119; and (4) provide a contact person assigned by the Commissioner's Office who will answer your phone calls and messages.

We have reviewed the information in the petition and the administrative record. We have determined that FDA has adequately considered and responded to your requests regarding filing your comment on the CCSVI procedure, receiving a formal response to your comment by telephone, and receiving a contact person who will answer your calls and messages. You have made these requests multiple times in a variety of submissions to FDA, and Center for Devices and Radiological Health (CDRH) former Ombudsman David Buckles and Associate Commissioner Leslie Kux, among others, have answered them fully. We deny reconsideration of these three requests. On further review we find that we have not responded fully to petition FDA-2012-P-0119 and therefore have not previously considered this material in the administrative record. We have determined that your petition meets the other requirements of section 10.33(d), and therefore the Agency is granting your request for reconsideration only as it relates to your request for a response to petition FDA-2012-P-0119.

II. AGENCY RESPONSE

Your petition FDA-2012-P-0119 requested that the Agency: (1) issue a statement that the "liberation procedure" may benefit patients who have neurological problems; (2) create an

"'unapproved' drug[s] category that requires minimum FDA forms maybe one FDA form"; (3) permit all interested patients to participate in clinical trials, and let all participants receive the drugs being tested; (4) make "the drug identified as BB7075 as an AIDS treatment" available to U.S. citizens, and ask the NIH to test it; (5) permit patients to have more influence in drug approval decision-making; and (6) provide safety information from investigational new drug trials. We will address each of these issues below.

First Request

In the first item in Petition FDA-2012-P-0119, you request that the Agency issue a statement that the "liberation procedure" may benefit patients with certain neurological problems sometimes referred to as CCSVI. The petition presents information regarding the theory behind the procedure and certain anecdotal evidence about individuals who have had the procedure in support of the positive effects that the procedure can have. FDA responded fully to this issue in correspondence from the former CDRH Ombudsman to you dated September 15, 2012 (copy enclosed), and in its June 19, 2014, decision. As we informed you in our June 19, 2014 response FDA is not aware of any valid scientific evidence demonstrating that the "liberation procedure" is effective in treating multiple sclerosis. As you know FDA has received adverse event reports, including a fatal event, related to this procedure. As a result the Agency released its May 10, 2012 Safety Communication: Chronic Cerebrospinal Venous Insufficiency Treatment in Multiple Sclerosis Patients (http://www.fda.gov/medicaldevices/safety/alertsandnotices/ucm303318.htm).

Based on FDA's review of the scientific evidence, there is no evidence that the "liberation procedure" may be beneficial to patients; and as such, FDA will not issue a public statement about this procedure as you requested. However, your petitions, supplements, and comments about CCSVI have been posted on the www.regulations.gov website and are available to the public. Accordingly, this request is denied and we will not address this issue further.

Second and Third Requests

Your Petition FDA-2012-P-0119 also requested that FDA create an "unapproved drug" category and permit all interested patients to participate in clinical trials and receive the drugs being tested. More specifically, the 2012 petition asserted that "there should be an 'unapproved' drug[s] category that requires minimum FDA forms maybe one FDA form" and "people taking the drug should understand that the drug is 'unapproved' and it is truly investigative." The 2012 petition also argued that FDA should provide "more humane alternatives" than a clinical trial with reduced requirements for participants.

The access to unapproved drugs that you requested is provided through FDA's Expanded Access program. FDA has a long history of facilitating access to investigational drugs for treatment use for patients with serious or immediately life-threatening diseases or conditions for which there are no comparable or satisfactory therapeutic alternatives. FDA revised its Expanded Access regulations in 2009 to clarify this program and issued a final *Guidance for Industry: Expanded Access to Investigational Drugs for Treatment Use — Qs & As* in June 2016. The guidance may be found at

http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm351261.pdf.

FDA's regulations allow access to investigational drugs in a wide range of circumstances, including access by individual patients.[2] Expanded access to investigational drugs is available to patients who have a serious or life-threatening condition when no other comparable or satisfactory treatment options are available.[3] FDA may permit an individual patient to access an investigational drug if the patient's physician determines that the probable risk to the patient from the investigational drug is not greater than the probable risk from the disease or condition, provided other applicable requirements are met. The Petition FDA-2012-P-0119 suggested that FDA reduce the paperwork required for access to investigational drugs. In fact, FDA may authorize Expanded Access for an individual patient without any written submission if there is "an emergency that requires the patient to be treated before a written submission can be made."[4] Authorization for such emergency situations is often provided over the telephone. Recently, FDA made available a revised form that provides a streamlined method for doctors to request expanded access to an investigational drug for individual patients. The form is much shorter and easier for doctors to use and takes much less time to fill out. The availability of the new form was announced in FDA guidance entitled "Individual Patient Expanded Access Applications: Form FDA 3926". The guidance may be found at http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM432717.pdf. Accordingly this request is granted.

Fourth Request

Your Petition FDA-2012-P-0119 also requested that FDA make "the drug identified as BB7075 as an AIDS treatment" available to U.S. citizens and ask the National Institutes of Health (NIH) to test the drug.

There is limited public information about this drug. Dr. Gary Davis stated publicly that he developed this product, sometimes referred to as goat neutralizing antibody drug, as a treatment for AIDS and that he was willing to provide it to patients infected with the AIDS virus. Dr. Davis, however, died in 2007, and there is no public information available indicating that another person or entity took over the development of the drug after his death. Your request regarding patient access to this drug is denied.

You also request that FDA ask NIH to test this product. NIH is a federal agency that, among other things, funds biomedical research. NIH is independent of FDA and establishes its own research priorities. It is not FDA's prerogative to make this request to NIH. Therefore, your request that FDA ask NIH to test BB:7075 is denied.

Fifth Request

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[2] 21 CFR 312.310.

[3] *Id.* at 312.305(a)(1).

[4] 21 CFR 312.310(d). We note that in such situations the patient's physician must agree to submit an expanded access submission within 15 working days of FDA's authorization of the use.

Petition FDA-2012-P-0119 further requested that FDA permit patients to have more influence in drug approval decision-making. The Agency has been working to increase the involvement of patients in the drug development process for some years.

In 2012, FDA began a new initiative called Patient-Focused Drug Development (PFDD) with the goal of obtaining the patient perspective on the drug approval process.[5] Patients who live with a disease have a direct stake in the outcome of the drug development process and are in a unique position to contribute to weighing benefit-risk considerations that can occur throughout that process. FDA is committed to obtaining input from patients concerning the impact of the disease, the spectrum of severity for those who have the disease, the measures of benefit that matter most to patients, and the adequacy of the existing treatment options.

Starting in 2013, FDA began hosting patients at a series of public meetings and webcasts, each focused on obtaining their perspectives on a specific disease.[6] To date, FDA has conducted patient meetings open to the public concerning such diseases as sickle cell disease, fibromyalgia, pulmonary arterial hypertension, neurological manifestations of inborn errors of metabolism, hemophilia and heritable bleeding disorders, and idiopathic pulmonary fibrosis. The series continues in 2016 with a series of meetings and webcasts concerning other conditions. Prior to each meeting, FDA opens a docket to accept comments from patients who are unable to attend in person or participate in the webcast. More information about these meetings and FDA's PFDD initiative can be found at http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm368342.htm. FDA is committed to advancing this important initiative and shortly will begin developing methodologies for collecting reliable, accurate and representative patient experience data so that it can be incorporated into the process of reviewing new drugs. For a description of the Agency's continued work in this area please go to the discussion that begins on page 27 of the PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2018 Through 2022 which can be accessed at http://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM511438.pdf. Given the Agency's work in this area, your request that patients be more involved in the process of evaluating drugs is granted.

Sixth Request

Your Petition FDA-2012-P-0119 also requested that FDA make safety information about an investigational drug available to "a person that has used a drug" and cites 21 CFR 601.50 as the source of the agency's obligation to do so. It appears that you believe this regulation creates a general requirement to provide safety information about an investigational product to any member of the public who requests it.[7] This is a misunderstanding of this regulation which is narrower and permits the agency to disclose an Investigational New Drug application (IND)

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[5] FDA, Prescription Drug User Fee Act V; Patient-Focused Drug Development; Consultation Meetings; Request for Notification of Patient Stakeholder Intention To Participate, 77 FR 55848 - 50 (Sep. 24, 2012).
[6] Patient-Focused Drug Development: Disease Area Meetings Planned for Fiscal Years 2013-2015, http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm (last visited: Oct. 6, 2014).
[7] The regulation cited in the petition relates to biological products. There is a similar provision for drugs at 21 CFR 312.130.

adverse reaction report to an individual on whom an investigational biological product has been used relating to such use upon request. Thus, the requester must be a person on whom the investigational biologic has been used, and cannot be a general member of the public. FDA does comply with this provision of the regulations when a request is made. Otherwise, during the investigational stage, disclosure of information from an IND is limited.[8] The Agency is often required to disclose an action package, which is the compilation of the FDA scientific review summaries, approved labeling, adverse reaction reports, and other important documents subsequent to approval.[9]

In addition, the safety information regarding an approved drug is included in the FDA-approved physician prescribing information which is publicly available. Safety information is also distributed to the public in Drug Safety Communications. This information can be found at http://www.fda.gov/Drugs/DrugSafety/ucm199082.htm. Thus, there are a number of avenues for patients and consumers to obtain drug safety information. Therefore your request that safety information about FDA-regulated drugs be released is granted.

III. CONCLUSION

You made four requests in your petition and stated: (1) "I would still like my comment to be included in the FDA folder for the procedure CCSVI"; (2) "I would like my first citizen petition # FDA-2012-P-0119 addressed and answered"; (3) "I would like the CDRH to formally answer my positive comment by telephone"; and, (4) "I would like the Commissioner's office to issue me a contact that will contact me and answer my phone calls and my messages."[10]

FDA has responded to each of these requests. As described in the Agency's June 19, 2014, decision, your comments concerning CCSVI have been forwarded to FDA's Executive Secretariat and are publicly available on the www.regulations.gov Internet website. In addition, FDA has reconsidered its June 19, 2014, decision and has provided a complete answer to Petition FDA-2012-P-0119 as described above. Given that FDA substantively responded to your various concerns, further discussion of this matter is not warranted. The Agency, therefore, respectfully declines your requests for further telephone calls with CDRH and a designated contact in the Commissioner's office. Further, FDA considers these matters closed and does not intend to respond to future correspondence regarding these issues.

Sincerely,

Leslie Kux
Associate Commissioner for Policy

[8] *See* 21 CFR 312.130, 601.50.
[9] *See* section 505(l) of the FD&C Act.
[10] Appeal at 3 (capitalization changed to sentence case).