UNIVERSITY OF PENNSYLVANIA MEDICAL CENTER

DEPARTMENT OF ORTHOPAEDIC SURGERY

The Children's Hospital of Philadelphia
Center for Bone Health

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January 19, 2018

Re: Patient: Luka Khosriashvili (DOB: 5/23/04)
Mother: Anna Kherumovi (DOB: 10/25/80)
Father: Konstantin Khosriashvili (DOB: 02/26/73)
Sister: Elene Khosriashvili (DOB: 12/29/09)
Address: Ketevan Tsamebulis AV– Apt. 39, Tbilisi City 0144, Republic of Georgia.

To Whom It May Concern:

We are writing this letter to strongly support the application of a visa for Luka Khosriashvili and his family to relocate from the Republic of Georgia and reside in the United States for a two-year period.

Luka is my patient, a 13-year-old boy diagnosed with an extremely rare and progressively disabling genetic condition called fibrodysplasia ossificans progressiva (FOP). FOP is a disorder characterized by congenital malformation of small joints and trauma-induced or spontaneous, episodic painful, inflammatory flare-ups that turns muscle and tendons into bone leading to immobility of all major joints of the axial and appendicular skeleton, rendering movement impossible. Luka is one of approximately 800 known patients with FOP worldwide and has debilitating manifestations of the disease. Specifically, FOP causes permanent physical disability which is not due to normal aging, is a very serious condition that causes significant difficulties in daily life. Currently, there are no disease-modifying medications, and the standard of care includes anti-inflammatory medications, which he currently uses as prescribed.

Luka was diagnosed with FOP at the age of 10. He is affected with FOP in his neck, spine, and both shoulders. His is unable to lift his arms above his head, and requires assistance with lifting and carrying, dressing, and activities of daily living. He is able to feed himself. He has difficulty with walking. He experiences pain at and near affected joints, and becomes easily fatigued with doing even basic activities. The reduced mobility substantially increases his risk for falls, and the likelihood of injuries that precipitate additional flare-ups that ultimately lead to locked joints. Luka's FOP condition will be progressively deteriorating, and will eventually result in his inability to function independently.

Clementia Pharmaceuticals have been conducting clinical trials in FOP at the University of Pennsylvania, and will be starting a Phase III trial early 2018 at limited sites including The Children's Hospital of Philadelphia. In order for Luka to participate in the Phase III Clementia Clinical Trials, he would require to reside in the USA for the two years' duration of the study.

The family will be responsible for their own medical health insurance coverage that would cover all expenses not related to the study. Travel and accommodation and all living expenses will be the responsibility of the family. Expenses for study-related office visits and tests while enrolled in the clinical trials would be covered by Clementia.

For more information on the clinical trials please visit the clinical trial website at: https://clinicaltrials.gov/ct2/show/study/NCT03312634. For more information on FOP, please visit the FOP website at: www.ifopa.org

We thank you in advance for approving the visa application to allow Luka and his family to reside in the United States for two years' duration while he is enrolled in the clinical trials.

If you have any questions, please call me at 1-267-303-6511; or email me at: Mona.AlMukaddam@uphs.upenn.edu.

With many thanks.

Sincerely yours,

Mona Al Mukaddam, MD, MS
Ian Cali Clinical & Research Scholar
Center for Research in FOP & Related Bone Disorders;
The Perelman School of Medicine
The University of Pennsylvania
Principal Investigator: Clementia Clinical Trials

Edna Mancilla, MD
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