



1600 20th Street, NW • Washington, D.C. 20009 • 202/588-1000 • www.citizen.org

Making Publicly Funded Medical Technologies Accessible in Developing Countries **Sickle Cell Disease**

Ensuring Access to Aes-103 – Leading Sickle Cell Disease Treatment in Development

Sickle Cell Disease: More than 300,000 people are born with Sickle Cell Disease (SCD) each year¹; more than 75% of which are in Sub-Saharan Africa², making it the most prevalent genetic disease in the World Health Organization (WHO) African Region³. An estimated 28,640 people die from SCD each year, more than 97 percent of which live in developing countries⁴. 44% of SCD mortality occurs in children under five years old.⁵ A 2006 report by the World Health Organization found that “sickle-cell anaemia contributes the equivalent of 5% of under-five deaths on the African continent, more than 9% of such deaths in west Africa, and up to 16% of under-five deaths in individual west African countries.”⁶

There are numerous measures that could be taken to decrease the burden of SCD in Sub-Saharan Africa. (For example, Benin has implemented measures including neo-natal screening and comprehensive health care management which has led to a significant decrease in the under-five mortality rate.⁷) However, a 2010 WHO report states: “Most countries have inadequate national health policies and plans, and scarce facilities, diagnostic tools, treatment services and trained personnel. There is therefore a need for urgent interventions to address this public health problem.”⁸

Even in the developed world, there is no widely accepted clinical cure for SCD.⁹ In the U.S., the only Food and Drug Administration (FDA) approved SCD treatment is hydroxyurea – and this is only indicated for use in adults, is only moderately effective and has undesirable side effects.¹⁰

Aes-103: Aes-103 has the potential to be a game changer for treatment of SCD. Currently in Phase II clinical trials, Aes-103 is being developed for use in children and adults to prevent the sickling of red

¹ Piel, F., Patil, A., Howes, R., Nyangiri, O., Gething, P., Dewi, M., ... Hay, S. (2012). Global epidemiology of sickle haemoglobin in neonates: A contemporary geostatistical model-based map and population estimates. *The Lancet*, 381, 142-151. Retrieved October 28, 2014, from <http://www.thelancet.com/pdfs/journals/lancet/PIIS014067361261229X.pdf>

² Ohene - Frempong, K. (2013, October 8). Global Burden of Sickle Cell Disease. Retrieved October 28, 2014, from http://www.sicklecelldisease.org/sicklecelldisease/uploads/File/2013_Convention_PowerPoint_Presentations/Ohene-Frempong-SCDAA-Convention-Sep-2013_copy.pdf

³ World Health Organization Regional Office for Africa. (2010, June). *Sickle-Cell Disease: A Strategy for the WHO African Region* (AFR/RC60/8). Retrieved from the WHO African Region website: http://www.afro.who.int/index.php?option=com_docman&task=doc_download&gid=5436

⁴ Institute for Health Metrics and Evaluation (IHME). GBD Database. Seattle, WA: IHME, University of Washington, 2014. Available from <http://www.healthdata.org/search-gbd-data?s=sickle%20cell%20mortality>. (Accessed October 28, 2014.)

⁵ *Ibid.*

⁶ World Health Organization. (2006, April). *Sickle-cell anaemia* (A59/9). Retrieved from WHO website: http://apps.who.int/gb/archive/pdf_files/WHA59/A59_9-en.pdf

⁷ *Ibid.* 3

⁸ *Ibid.* 3

⁹ *Ibid.* 3

¹⁰ Aes-103 for Sickle Cell Disease. Retrieved October 28, 2014, from <http://www.ncats.nih.gov/research/rare-diseases/trnd/projects/scd.html>

blood cells by “maintaining the abnormal hemoglobin molecule in a conformation that does not undergo polymerization and sickling.”¹¹

The compound Aes-103 was developed at Virginia Commonwealth University by a team led by Dr. Donald Abraham through funds granted by the National Heart, Blood, and Lung Institute (NHBLI), one of the bodies of the National Institutes of Health (NIH).¹² Dr. Abraham and another member of his team, Dr. Martin Safo, received a combined \$1,305,764¹³ through NHBLI grants listed on the compound’s patent from the years 2000 through 2004. An additional patent on Aes-103 lists a separate NIH grant to Dr. Abraham, totaling \$2,369,074¹⁴ from the years 1991-2000.

A search for “Aes-103” in the NIH’s Research Portfolio Online Reporting Tools database (RePORT) shows six additional grants towards the development of the drug. Two of these grants went to the NIH’s National Center for Advancing Translational Sciences for multiple projects¹⁵, of which work involving Aes-103 was only a component, and granular financial information is not readily available for these grants. One of the grants focused on SCD¹⁶, but with a wider focus than only Aes-103. The other three grants specifically funded Phase I clinical trials¹⁷, as well as the ongoing Phase II clinical trials¹⁸ for the drug. NIH funding of Aes-103 Phase I trials totaled \$1,055,422, and while funding information for Phase II trials is not yet available through RePORT, a release¹⁹ from the FDA states the grant will be worth approximately \$1.6 million over four years.

Despite the public’s role in the discovery of Aes-103 and its ongoing role in the clinical development of the drug, Aes-103 is now owned by the biopharmaceutical firm Baxter International Inc.²⁰ The U.S. government can exercise its rights in Aes-103 to ensure cost does not become a barrier to widespread access where it is needed most.

¹¹ *Ibid.*

¹² Peters, E. (2014, July 9). Another step toward sickle cell disease treatment. Retrieved October 28, 2014, from http://news.vcu.edu/article/Another_step_toward_sickle_cell_disease_treatment

¹³ *See*

http://projectreporter.nih.gov/project_info_details.cfm?aid=6526574&icde=22316786&ddparam=&ddvalue=&ddsub=&cr=1&cs=b=default&cs=ASC&MMOpt= and

http://projectreporter.nih.gov/project_info_details.cfm?aid=6782522&icde=22317036&ddparam=&ddvalue=&ddsub=&cr=1&cs=b=default&cs=ASC&MMOpt=

¹⁴ *See*

http://projectreporter.nih.gov/project_info_details.cfm?aid=6183131&icde=22317109&ddparam=&ddvalue=&ddsub=&cr=1&cs=b=default&cs=ASC&MMOpt=

¹⁵ *See* http://projectreporter.nih.gov/project_info_details.cfm?aid=8557104&icde=22324863 and

http://projectreporter.nih.gov/project_info_details.cfm?aid=8752453&icde=22324863&ddparam=&ddvalue=&ddsub=&cr=4&cs=b=default&cs=ASC&MMOpt=

¹⁶ *See*

http://projectreporter.nih.gov/project_info_details.cfm?aid=8762091&icde=22324863&ddparam=&ddvalue=&ddsub=&cr=5&cs=b=default&cs=ASC&MMOpt=

¹⁷ *See* http://projectreporter.nih.gov/project_info_details.cfm?aid=8746698&icde=22324863 and

http://projectreporter.nih.gov/project_info_details.cfm?aid=8558082&icde=22324863

¹⁸ *See* http://projectreporter.nih.gov/project_info_details.cfm?aid=8751774&icde=22324863

¹⁹ FDA awards grants to stimulate drug, device development for rare diseases. (2014, September 30). Retrieved October 28, 2014, from <http://www.fda.gov/newsevents/newsroom/pressannouncements/ucm416738.htm>

²⁰ AesRx – Biopharmaceutical Company Targeting Sickle Cell Disease. (2014, July 9). Retrieved October 28, 2014, from <http://aesrx.com/>