

# Dei BioPharma's new gene therapy offers hope for sickle cell treatment

By John Odyek

Dei BioPharma Limited has announced the development of a new technological approach aimed at treating sickle cell disease.

The biotechnology company that is focused on affordable next-generation medicines developed a promising Clustered Regularly Interspaced Short Palindromic Repeats-based gene therapy platform for sickle cell disease.

Gene therapy is a new scientific technique that modifies a person's genetic material to treat or prevent disease, offers new hope by replacing faulty genes, disabling harmful ones or introducing new ones into the body.

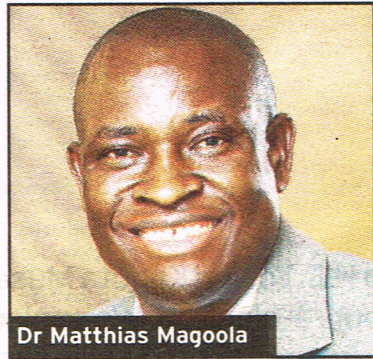
The approach was invented by the company's founder and chief

executive officer, Dr Matthias Magoola. It is designed to offer a universal treatment approach that could significantly lower the cost and complexity of gene therapy for people living with sickle cell disease.

Unlike the existing gene therapies that must be customised for individual patients or specific genetic mutations, Dei BioPharma's approach targets a shared genetic switch that controls the production of foetal haemoglobin, a statement from the company said, yesterday.

Magoola said by reactivating foetal haemoglobin – which protects red blood cells from sickling – the therapy aims at reducing disease severity across all forms of sickle cell disease.

He said the therapy works by editing a regulatory element of the BCL11A



Dr Matthias Magoola

gene, which plays a key role in the natural transition from foetal to adult haemoglobin after birth. Higher levels of foetal haemoglobin are widely associated with fewer complications and milder symptoms in sickle cell patients, Magoola said.

"This invention was designed to address not only the biology of sickle cell disease, but also the challenge of access," Magoola said.

He said targeting a universal genetic switch rather than the sickle mutation itself, there can be development of a single, standardised gene-editing product that could be used for all patients.

"By targeting a universal genetic switch rather than the sickle mutation itself, we can manufacture a single, standardised gene-editing product applicable to all patients. This opens the door to what we believe can become the first scalable, first-in-line generic gene therapy platform for a monogenic disease," Magoola said.

With over 400,000 Ugandans affected by sickle cell disease, demand for advanced therapy is significant.

Uganda ranks fourth globally in sickle cell prevalence according to the Ministry of Health. Gene therapy offers long-term or permanent solutions by targeting diseases at genetic level. In advanced economies, such therapies are already curing some forms of sickle cell disease and HIV through gene editing and stem cell techniques.

Dr Cissy Kityo Mutuluza, the executive director of the Joint Clinical Research Centre, said a product to cure sickle cell disease now exists in the US and Europe, but costs up to \$2.5m (about sh9b) per patient. The global gene therapy industry is projected to surpass \$10b.

"With the right infrastructure and policy, Uganda can lead the continent in affordable, locally adapted gene therapies," Mutuluza said.