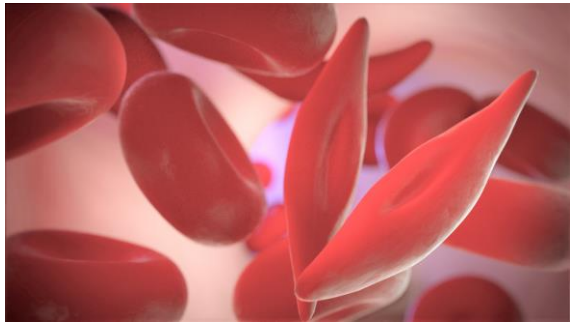
OXFORD UNIVERSITY INNOVATION

Base editing proteins to repair mutations in the haemoglobin gene

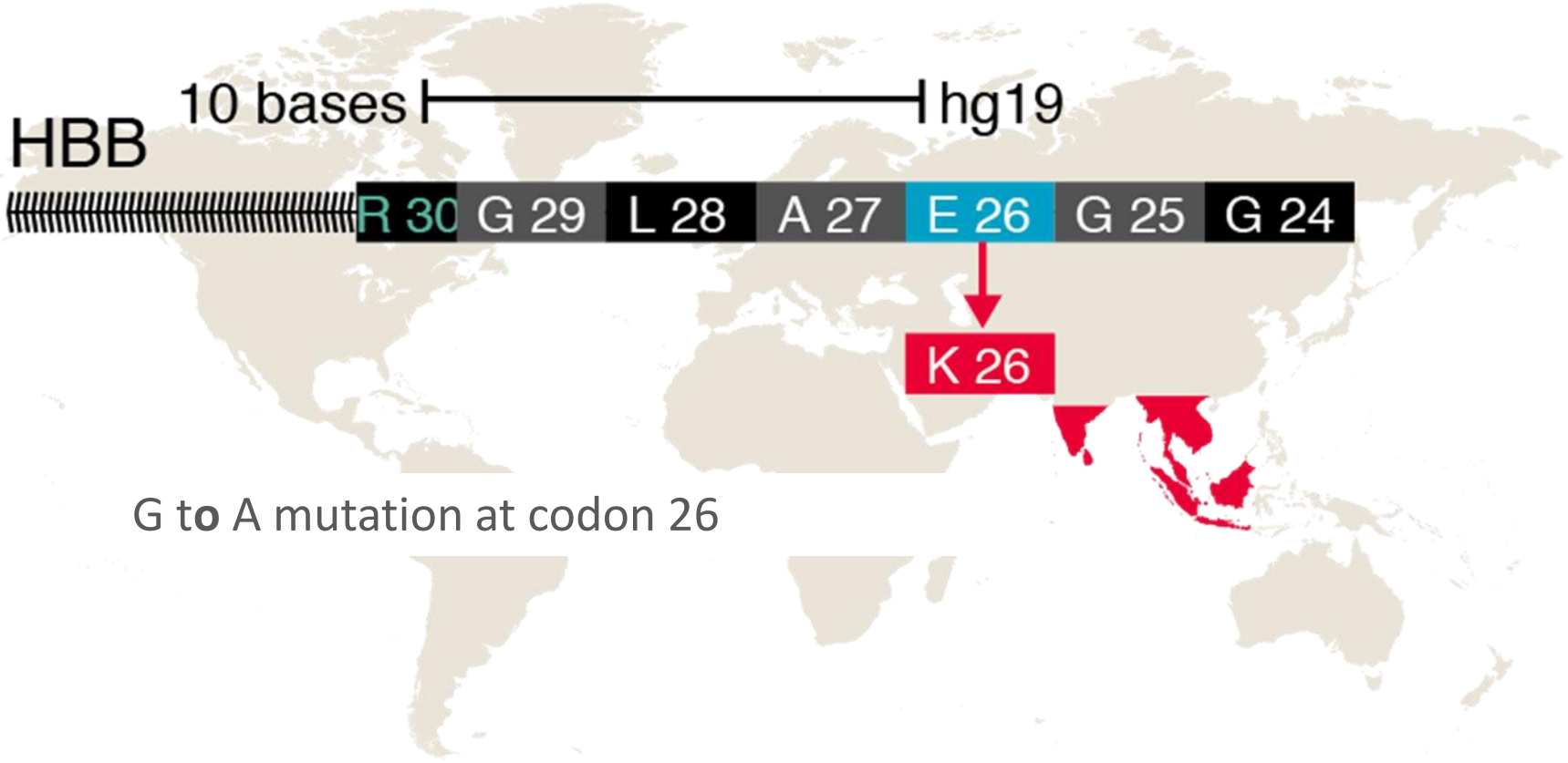
OUI Project 16008

Haemoglobinopathies

- inherited disorders caused by mutations in human beta globin gene
- Haemoglobin E (HbE, beta thalassaemia):
  - high prevalence in Asia – confers resistance to malaria
  - causes severe thalassaemia in combination with second mutation
  - combination causes 50% of severe thalassemia worldwide (20,000 births p.a.)
- Haemoglobin S (HbS, sickle cell disease)
  - Severe congenital anaemia
  - Life-threatening complications
  - Median life expectancy approx. 60yrs (many die in their 40s)



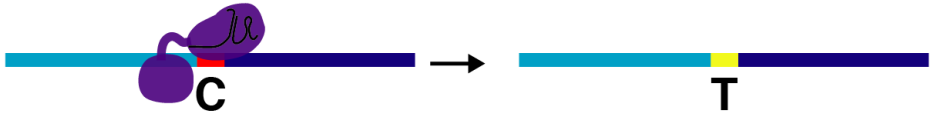
HbE beta thalassaemia



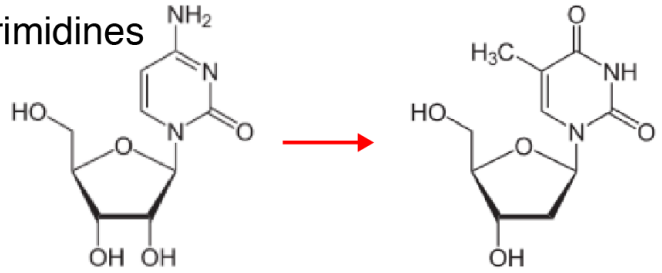
G t**o** A mutation at codon 26

Base editing

Cytidine Deaminase (BE3)



Pyrimidines

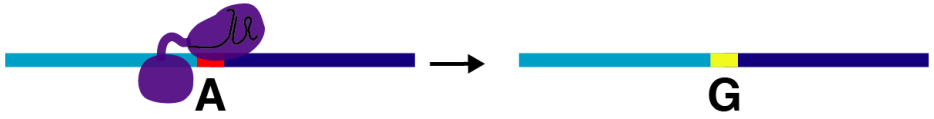


Cytosine

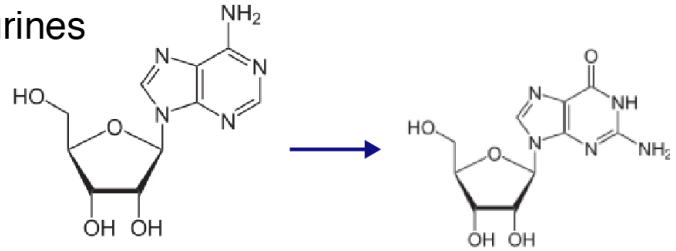
Thymine

Komor et al., 2016

Adenine Deaminase (ABE 7.10)



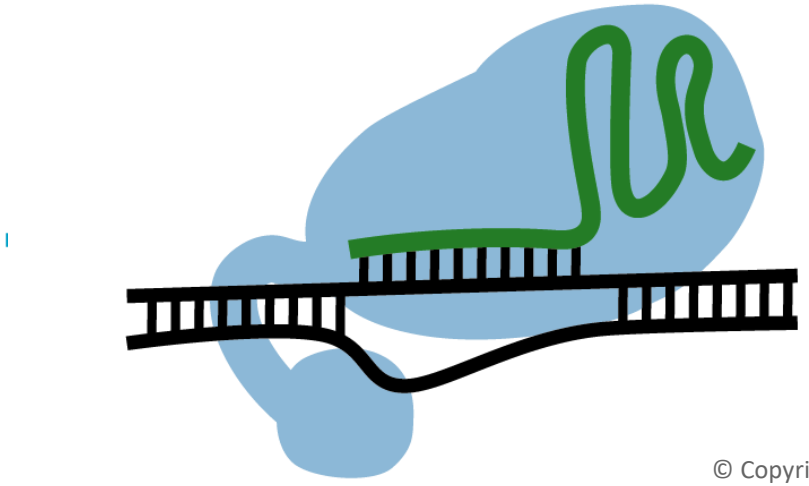
Purines



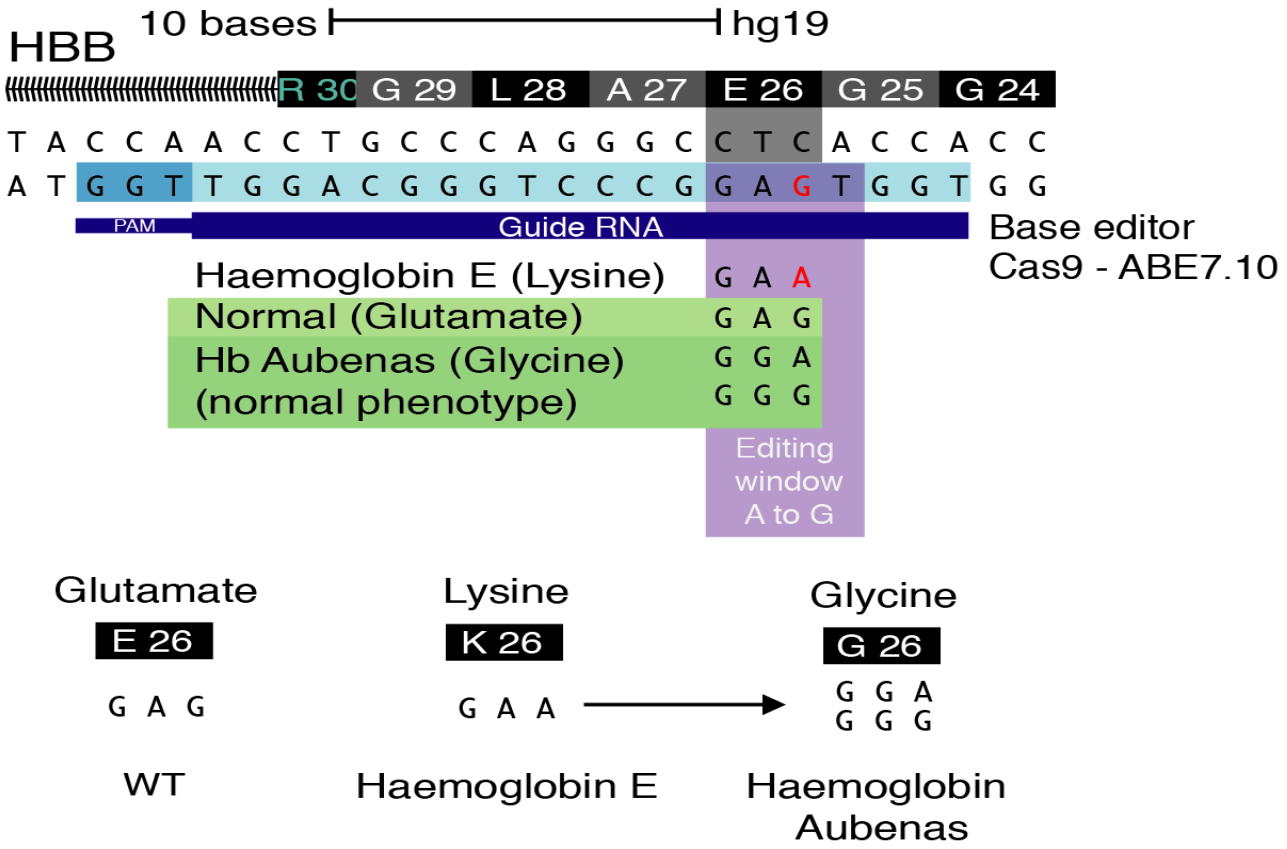
Adenine

Guanine

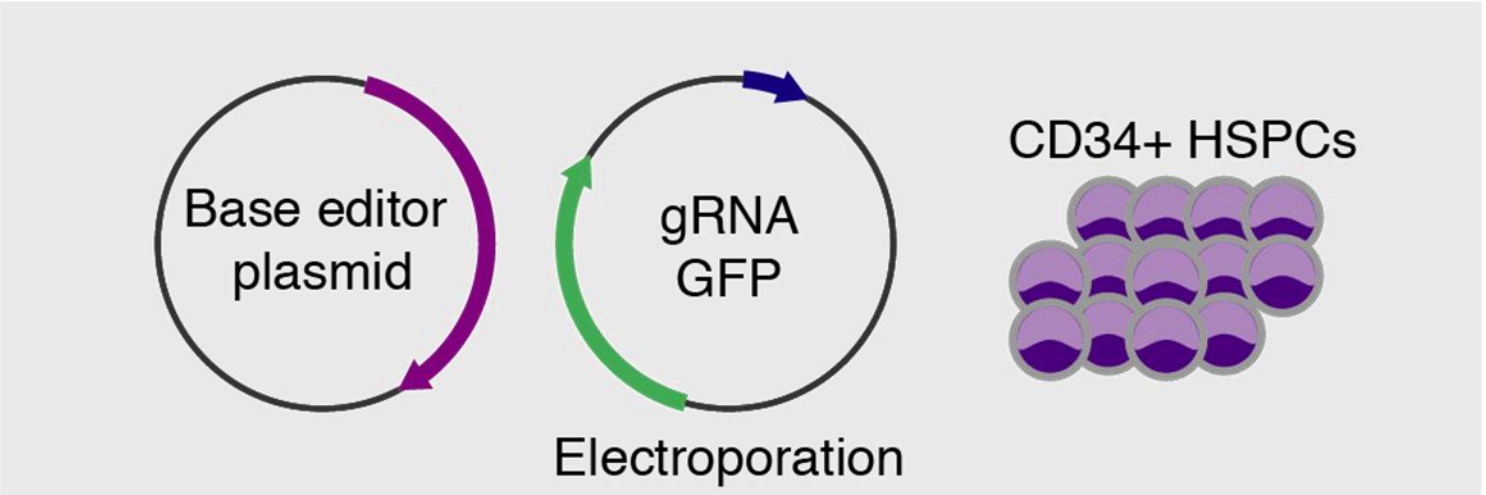
Gaudelli et al., 2017



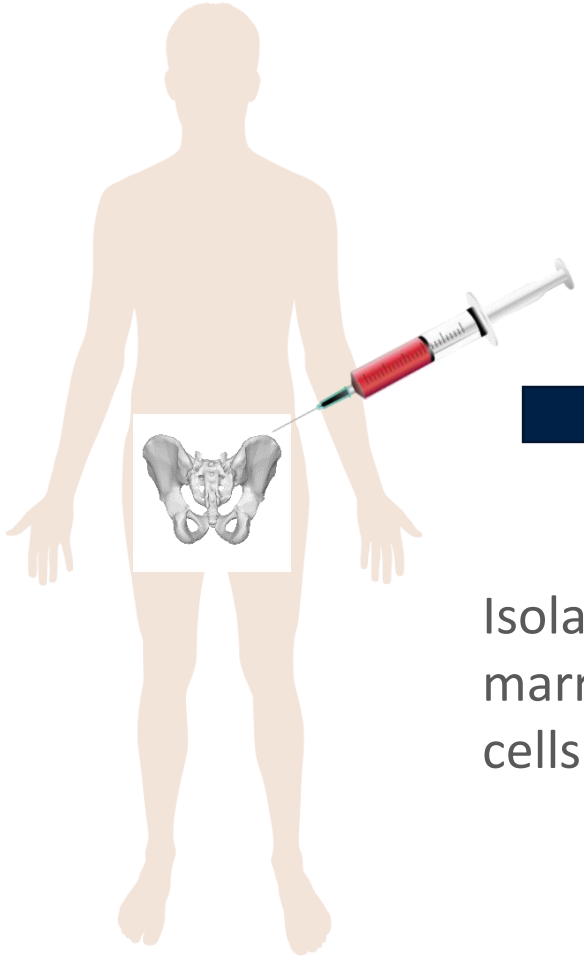
Oxford invention HbE



Editing HbE in CD34+ Haemopoietic stem cells



Autologous bone marrow transplant

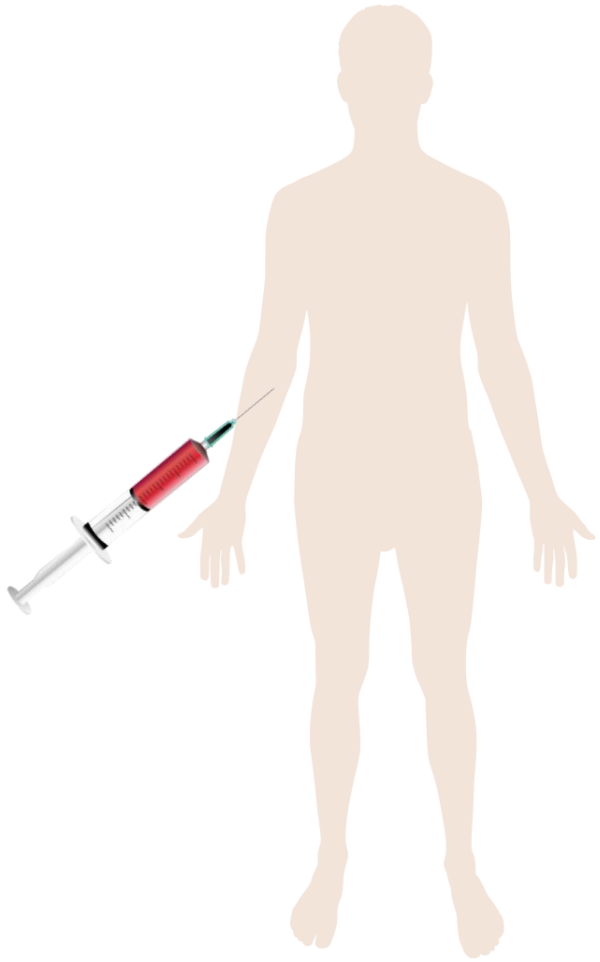


Transfusion dependent

Isolate bone marrow stem cells

Base Edit

Reinfuse



Normal

Summary

- Significant unmet need
- Benefits over competing approaches (e.g. gene therapies, gene editing)
- Preclinical stage project developed by Clinician Scientist/Consultant Haematologist (stem cell transplantation)
- International Patent Application WO/2020/065303 “EDITING OF HAEMOGLOBIN GENES” (filing date 25.09.2019)
- Requires access to a suitable base editing platform
- Commercial partner sought for further development & exploitation

Any questions?

Contact:

Dr Matthew Carpenter
Principal Licensing & Ventures Manager
Matthew.carpenter@innovation.ox.ac.uk
Please quote project reference OUI16008

www.innovation.ox.ac.uk

linkedin.com/company/oxford-university-innovation

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