Molecular Basis for Sickle Cell Disease
Adapted from Nicholas’ Story by Didem Vardar-Ulu for CH373-S20

POST-CLASS WORK- VERSION B

Q2A. (10 pnts) In the video you watched, Nicholas says hydroxyurea an approved drug for treating Sickle Cell Disease, changed his life. His mother explained that since he started the hydroxyurea treatment, Nicholas has been able to be more active and have a regular schedule with sports, school, and friends.

“What does hydroxyurea do and how does it help Nicholas have a more regular life?”

Search for hydroxyurea in DrugBank (https://www.drugbank.ca/drugs/DB01005), a curated resource that provides a wide variety of information of drugs and drug-like molecules. Using the information provided about what the drug does and its mechanism of action and your understanding of the molecular basis of sickle cell disease write a short explanation of how hydroxyurea help SCD patients. Your answer should provide a plausible explanation to how the interferes with the sickling process. Make sure to provide specific biochemical links between what the drug’s direct effect is and the ultimate outcome (improvement in symptoms) based on the molecular explorations you did during the “in-class” part of the case study. You will be graded based on the correct and specific biochemistry you offer in your hypothesis and not for “the correct answer”.

Q2B. (10 pnts) In November 2019 the FDA approved voxelotor for adults and pediatric patients 12 years of age and older with sickle cell disease.

Search for voxelotor in DrugBank (https://www.drugbank.ca/drugs/DB14975), a curated resource that provides a wide variety of information of drugs and drug-like molecules. Using the information provided about what the drug does and its mechanism of action and your understanding of the molecular basis of sickle cell disease write a short explanation of how voxelotor help SCD patients. Your answer should provide a plausible explanation to how the drug interferes with the sickling process. Make sure to provide specific biochemical links between what the drug’s direct effect is and the ultimate outcome (improvement in symptoms) based on the molecular explorations you did during the “in-class” part of the case study. You will be graded based on the correct and specific biochemistry you offer in your hypothesis and not for “the correct answer”.

You can also access a journal article reporting Pharmacokinetics and pharmacodynamics of voxelotor through the following link: https://drive.google.com/file/d/16GmyO9bIO3ITYjG04YejMI9wyGCD_f6X/view?usp=sharing