**Grant Impact Report**

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| Name | Kevin Rouault-Pierre |
| Job title | Senior Lecturer |
| Organisation | Barts Cancer institute, QMUL |
| Co-investigators (if applicable) | N/A |
| Grant awarded | £20,000 |
| Year awarded | 2021 |
| Date started | 1st January 2022 |
| Date completed | 31st December 2022 |
| Total amount expended (£) | £20,000 |

This is the BSH grant impact report form. Please enter the full grant details above, and fill out the form below. The form should be completed electronically and sent to grants@b-s-h.org.uk. Please note that the report can only be accepted if all sections have been completed in full.

**In addition:** Please include a recent photo of yourself.

Your grant report and photo may be published in our communications materials, including our website and social media platforms.

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| 1. **Please summarise what the grant enabled you to achieve;** **what would not have been possible without the funding? (Up to 500 words)**

The BSH grant has been instrumental to complete a work that has recently been published in Science Translational Medicine (Mian&Philippe et al., 2023).  |
| 1. **Briefly describe the aims and intended outcomes of this project. Please clearly indicate if there is any sensitive information in this report that should remain confidential for now. (Up to 300 words)**

The aims of the BSH grant were (1) to characterise aberrant transcripts in *SF3B1*-MDS, (2) to validate these aberrant transcripts in validation cohorts and surrogate models and finally (3) to assess the functional impact of these aberrant transcripts in normal and malignant primary haematopoietic cells.  |
| 1. **Describe the key outcomes to date, including whether this grant has resulted in further research. Please summarise your conclusions**. **(Up to 600 words)**

The BSH grant has been instrumental to complete a work that has recently been published in Science Translational Medicine (Mian&Philippe et al., 2023). This work has been presented at key meetings in the field of Haemato-Oncology ESH-MDS, ASH (myeloid workshop and Biology of MDS session), MDS Foundation annual symposium and French club of Hematopoiesis.Building upon this recent study elucidating a promising treatment avenue for patients afflicted with myelodysplastic syndromes and anaemia, I am collaborating with Dr. Onima Choudhury, a clinical scientist at the University of Oxford, UK. Together, we endeavor to secure funding to develop a Phase 1B Clinical trial. This trial will aim to evaluate, through a dose escalation approach, the efficacy and toxicity of Vitamin B5 in *SF3B1*-MDS. |
| 1. **List published papers, oral and/or poster presentations as a result of this grant.Include manuscripts in preparation or in submission / under review, prefaced by an asterisk.**

**Manuscript:**[Vitamin B5 and succinyl-CoA improve ineffective erythropoiesis in SF3B1-mutated myelodysplasia.](https://pubmed.ncbi.nlm.nih.gov/36857430/) Mian SA\*, Philippe C\*, Maniati E, Protopapa P, Bergot T, Piganeau M, Nemkov T, Di Bella D, Morales V, Finch AJ, D'Alessandro A, Bianchi K, Wang J, Gallipoli P, Kordasti S, Kubasch AS, Cross M, Platzbecker U, Wiseman DH, Bonnet D, Bernard DG, Gribben JG, **Rouault-Pierre K#.** Sci Transl Med. 2023 Mar;15(685):eabn5135. doi: 10.1126/scitranslmed.abn5135. Epub 2023 Mar 1.PMID: 36857430**List of oral presentations on the work presented above:**From the group:2023 May: oral communication the 17th International Congress on Myelodysplastic Syndromes (Celine Philippe Postdoctoral Fellow)  2022 April: oral communication ESH MDS (Celine Philippe Postdoctoral Fellow)  2022 January: Oral communication (KRP) at the RNA UK 2022  2021- December: Oral presentation by Celine Philippe (Postdoctoral Fellow) at the American Society of Hematology, USA  From KRP:2024- February – Invited keynote speaker, i4MDS EHA, The Hague, Netherland  2024- January – Speaker, CRUK City of London Symposium, London, UK  2023- November – Invited speaker Cambridge Stem Cell UK-China Molecular Haematopoiesis workshop, Tianjin, China  2023- October – Invited speaker, ICR seminar, London, UK  2023- September – Speaker, Editor Consortium meeting, Madrid, Spain  2023- July – Invited speaker, Stem Cell Lunch, KCL, UK  2022- October – Invited speaker NCRI MDS subgroup, UK  2022- June – Invited speaker Group of Francophone Myelodysplasia, Nantes, France  2021- December – Invited Speaker Myeloid Workshop American Society of Hematology congress, Atlanta, US  2021- November – Invited speaker 24th Molecular Haemopoieis meeting, London, UK   |
| 1. **Did any patent applications arise from this work? (If yes, please detail. Up to 200 words)**

Not applicable. |
| 1. **Were you successful in any further grant applications as a result of this work? (If yes, please detail. Up to 200 words)**

Since I was awarded the BSG grant, we have secured funding from CRUK, EHA and the Barts Charity on different other projects. |
| 1. **Did new collaborations arise from this work? (If yes, please detail. Up to 400 words)**

I am currently collaborating with Dr Onima Chowdhury to secure funding for a clinical trial building on the work developed in the publication mentioned above. |
| 1. **What was the funding amount you received and how was it actually spent? (detail item/activity and amount spent in pounds)**

The amount was £20,000 and was spent on consumables/sequencing dedicated to the project. |
| 1. **What are the future research priorities in this area?**

Low-risk MDS remains a disease group with significant unmet needs, compelling us to continue investigating the underlying molecular mechanisms driving its pathology. Our research focuses on identifying aberrant splicing events that can be targeted in the clinic, with the aim of either enhancing patients' quality of life or eradicating malignant clones. |