出國報告(短期研習)

## 主題:University College London Hospital Heamatology Visiting Elective Programme

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## 摘要

此次參與英國 UCLH 血液科的臨床實習,我深入學習了血液相關疾病的診斷與治療,包括白血病、淋巴瘤、多發性骨髓瘤等疾病的管理策略與操作技術。透過病房查房、門診觀摩及床邊教學,我增強了病史採集、診斷技能及與患者溝通的能力,並有機會觀察骨髓穿刺、PICC 線放置等操作過程。實習中,英國醫療體系中強調患者中心護理與分級醫療的模式深深啟發了我,特別是在與患者共同決策及推廣臨床試驗方面,展現出對個別化治療的重視。此外,在全英語環境中,我的專業英語能力與跨文化溝通能力得到顯著提升,進一步堅定了我成為具備國際視野與跨文化能力的醫療人才的目標。這段經歷讓我對醫學的專業深度與人文溫度有了更深層的體會,期許未來能在本地及國際醫療體系中提供高品質且以患者為核心的醫療服務。

關鍵字:血液科、臨床實習、患者中心照護、跨文化溝通、臨床試驗、國際醫療視野

#### 一、 目的

此次參與英國 UCLH 血液科的臨床實習,我的主要目的是深入學習血液相關疾病 的診斷與治療,並在實際操作中累積經驗。由於我在台灣的臨床實習中尚未接觸過血 液科,此次實習為我提供了一個寶貴的機會來彌補這方面的不足,特別是了解白血 病、淋巴瘤、骨髓增生性疾病以及其他血液疾病的完整治療流程。同時,我希望提升 自身病史收集、身體檢查及與患者溝通的能力,為未來從事醫師工作打下穩固基礎。

了解台灣與英國兩國醫療體系的差異亦是本次實習的重要目標之一。台灣醫療系 統以高效率和全民健保為特點,強調資源快速分配,而英國則注重分級醫療與全人照 護,並在病患的長期管理和臨床試驗中表現出優勢。我希望藉此機會比較兩者的不 同,探討在不同醫療環境下實現最佳患者照護的可能性,並將英國的一些經驗融入未 來的台灣臨床實踐中。

此外,這次實習也為我提供了拓展國際視野與提升軟實力的機會。在全英語環境 中與專業醫療團隊及患者互動,幫助我強化專業英語能力並克服語言挑戰。同時,這 樣的經歷讓我體會到文化差異對醫療實踐的影響,並進一步提升我的跨文化溝通能 力。作為一名醫學生,我期許自己未來能成為具備國際視野的醫療人才,不僅能勝任 本地工作,亦能積極參與國際醫療合作,為全球健康議題提供更多貢獻。

二、 過程

此次實習分為病房與門診兩個主要階段,每階段均安排了緊湊且針對性的活動, 讓我全面了解血液科的臨床運作模式。

#### 1. 病房實習

在病房實習中,我隨主治醫師與住院醫師進行每日查房,了解患者最新病情發展、 檢查結果與治療計劃。透過參與白血病、多發性骨髓瘤及淋巴瘤專科團隊的工作, 我觀察到醫師如何分析複雜病例並調整治療策略。同時,我見證了多學科協作在 提升患者照護質量中的關鍵作用。此外,病房實習也提供了觀察醫療操作的機會, 如骨髓穿刺與 PICC 線放置,讓我對 procedure 準備及後續護理有更直觀的認識。

#### 2. 教學活動

醫院每週安排的教學課程是實習的重要部分,包括基礎知識講解與床邊教學。課 程內容涵蓋化療副作用管理、自體造血幹細胞移植的適應症,以及骨髓增生性疾 病的診斷技術等等。床邊教學則強調實際操作,如患者檢查與病史採集,不僅提 升了我的臨床技能,也增強了我與患者的溝通能力。

#### 3. 門診實習

門診實習讓我接觸更廣泛的病例類型,涵蓋紅血球診所、骨髓增生性疾病診所與淋巴瘤診所。我主要協助記錄病史並進行基本檢查,同時觀察主治醫師為患者制

定個別化治療方案。在骨髓增生性疾病門診,我學習了 JAK 抑制劑的應用及其副 作用管理;在紅血球門診,則深入了解鐮狀細胞病與β地中海貧血的綜合管理策 略,並探索基因療法等未來治療方向。

三、 心得

此次在英國 UCLH 血液科的實習,對我的醫學生涯產生了深遠影響,不僅加深了 我對血液相關疾病的理解,也促使我重新思考如何在醫療工作中實現以患者為中心的 照護。實習期間,我深入參與了白血病、多發性骨髓瘤、淋巴瘤等疾病的診療過程, 學習到如何針對不同疾病制定個別化治療方案。在多發性骨髓瘤患者的病房管理中, 我觀察到醫師根據患者對治療的反應不斷調整化療藥物的劑量與搭配,確保治療的有 效性與安全性;而在淋巴瘤門診中,醫師透過病史、影像學結果與分期診斷,為患者 量身訂製治療計劃,從標準化療到臨床試驗藥物的選擇,每一步都體現了專業判斷與 細緻考量。這些臨床經驗彌補了我在台灣尚未接觸過血液科的學習空白,也增強了我 應對複雜病例的信心。

實習期間,我還有機會觀察並學習醫療操作過程,包括骨髓穿刺與 PICC 線放置 等技術。這些過程不僅讓我了解操作的準備工作與無菌處理的細節,也幫助我體會到 醫療程序中的精準與嚴謹是如何直接影響患者的預後與安全。此外,每周的教學活動 亦是實習的重要部分,透過基礎知識的系統講解與床邊教學的實際演練,我更深入理 解化療副作用的管理、自體造血幹細胞移植的適應症及其程序,並且在患者病史採集 和體檢中逐漸提高了操作熟練度與溝通能力。這些學習經歷讓我從理論到實踐都有了 顯著提升,對未來的醫學訓練奠定了更穩固的基礎。

值得一提的是,英國醫療體系中強調以患者為中心的照護,給我留下了深刻印 象。在門診觀察中,我看到醫師與患者詳細討論診斷結果及治療方案,並充分考慮患 者的期望和生活需求,這樣的互動不僅讓患者感受到醫療服務的溫度,也提升了治療 的依從性與成效。與台灣醫療系統相比,英國醫師能花更多時間與患者交流,而台灣 因患者量較大,診療往往強調效率。這樣的差異讓我意識到,盡管高效診療能提高資 源利用率,但若能在繁忙的環境中仍堅持患者中心的理念,將更有助於提升醫療品質 與患者滿意度。我特別受到啟發的是,醫師們在諮詢過程中展現的傾聽與耐心,這種 能力不僅體現了專業素養,也讓我明白醫學是幫助患者在生理與心理上都找到支持與 力量的藝術。

此外,我發現英國醫療系統非常注重臨床試驗的整合,許多血液科患者有機會參 與創新藥物的試驗,這不僅提供了更多治療選項,也為醫療進步注入動力。通過此次 實習,我學到了英國醫療在創新與臨床應用之間找到平衡的方式。同時,英國的分級 醫療制度也令我印象深刻,基層醫師在篩檢與轉診中的角色明確,極大程度地減輕了 專科醫師的負擔,確保每位患者都能在適當的階段接受對應的醫療資源。這樣的分工 合作模式對提升整體醫療效率具有重要啟示。 最後,這次實習也促進了我的個人成長,尤其在專業英語能力與跨文化溝通上有 了顯著提升。在全英語環境下與醫療團隊和患者互動初期,語言的挑戰確實讓我感到 壓力,但隨著時間的推移,我逐漸適應並學會以更清晰、簡單的方式表達專業意見, 並加強對醫學術語的掌握。同時,與不同文化背景的患者接觸,讓我深刻體會到醫療 並非單一標準化流程,而是需要根據文化差異進行靈活調整的工作。這段經歷不僅提 升了我的國際視野,也讓我更堅定未來要成為具備跨文化能力與國際移動力的醫療人 才的目標,無論是在本地醫療體系,還是在國際舞台上,都能提供高品質的醫療服務。

實習的每一天都充滿挑戰與收穫,從病房查房到門診觀摩,從教學課程到臨床操 作,我深刻感受到醫學的深度與廣度。透過這段經歷,我不僅學會了專業知識與技能, 更認識到醫療的核心是關懷與共情。



一、課程表

#### HAEMATOLOGY ELECTIVE TIMETABLE FOR CHUNG-WEI HSU 21 OCTOBER TO 15 NOVEMBER 2024

Report to: Drs Office, 2nd Floor, Grafton Way Building, Grafton Way for 09:00hrs



#### Ward Week from 21 to 25 October – Grafton Way Building

- Monday: Leukaemia Team Drs James Leveson, Mathilde Chanut, Sarah Mohamed
- Tuesday: Myeloma Team: Dr Constantinos Loukari
- Wednesday: Lymphoma Team Dr Dreen Gul
- Thursday: Red Cell Team Dr Usman Javed & Dr Andrea Diallo
- Friday: Myeloma Team Dr Constantinos Loukari

#### Clinic Week: Monday 28 to Thursday 31 October - Macmillan Cancer Centre (MCC)

MON 28 OCT	TUE 29 OCT		WED 30 OCT	THU 31 OCT
PM	AM	PM	AM	AM
1pm MPN Clinic	9am Haem Preg Clinic	1.30 pm WM Clinic	8.45am Gen Haem Clinic	9am Lymphoma Clinic
4th Floor MCC	EGA, Level 1	4th Floor MCC	4th Floor MCC	4th Floor MCC
Dr Jon Lambert	Dr Mari Thomas	Dr Shirley D'Sa	Dr Sajir Mohammedbhai	Dr Jon Lambert

#### Ward Week from 4 to 8 November - Grafton Way Building

- Monday: Leukaemia Team Drs Andrea Diallo, Elena Torre, Usman Javed
- Tuesday: Lymphoma Team Drs Cath Lecat, Derek Chan
- Wednesday: Red Cell Team Dr Chris Bailey
- Thursday: Myeloma Team Dr Vaishak Vidyadhar
- Friday: Leukaemia Team Drs Andrea Diallo, Usman Javed

#### Clinic Week: Tuesday 12 to Friday 15 November - Macmillan Cancer Centre (MCC)

<b>TUE 12 NOVEMBER</b>	WED 13 NOVEMBER	THU 14 NOVEMBER	FRI 15 NOVEMBER
AM	AM	AM	PM
8.30 am Red Cell Clinic 4th Floor MCC Dr Perla Eleftheriou	9am Myeloma Clinic 4th Floor MCC Dr Jonathan Sive	10am TYA Leukaemia 3rd Floor MCC Dr Jenny O'Nions	1.30pm Coag Clinic 4th Floor MCC Dr Matt Stubbs

二、考核表



三、實習報告(在英國每周撰寫並交由當地導師評核)

## Abstract

This elective in the hematology department at University College London Hospitals (UCLH) was my first hands-on experience with clinical hematology, a field I haven't yet explored in Taiwan. My goal was to build a strong foundation in assessing patients, using diagnostic techniques, and understanding treatment methods specific to hematology, while also improving my general internal medicine skills. Thanks to the excellent bedside teaching, I practiced important skills like taking patient histories and doing cardiovascular, chest, and abdominal exams. The practical and focused feedback I received has not only boosted my confidence in hematology but also made me more prepared for a wide range of internal medicine cases.

Ward rounds and clinic visits gave me a new perspective on patient communication and personalized care, made possible by a lighter patient load than what I'm used to in Taiwan. This setup allowed for detailed conversations about treatment options, possible side effects, and patient concerns, encouraging shared decision-making that could be carefully tailored to each individual. I also saw how the department's involvement in clinical trials added flexibility and creativity to treating challenging cases. Through rotations in specialties like leukemia, myeloma, lymphoma, and red cell disorders, with focused learning in each area, I gained a solid understanding of the wide range of conditions within hematology.

Reflecting on my experience, I noticed clear differences between healthcare in Taiwan and the UK, especially the role of General Practitioners (GPs), who play a key part in coordinating patient care. I was also impressed by the hospital's integrated systems that allow departments and even hospitals to work together seamlessly, which helps create well-rounded treatment plans that consider the patient's overall health. Although Taiwan's healthcare system has its own strengths, the values I saw here—listening closely to patients, explaining clearly, and working with them to make decisions—are principles I hope to bring back to my practice. This elective has not only deepened my knowledge of hematology but also strengthened my appreciation for patient-centered care, showing me how up-to-date treatments and compassionate communication can come together to provide high-quality, well-rounded care.

## **Learning Report**

Date: October 31 Department: Lymphoma Clinic

## **Patient Summaries and Learning Points**

#### Patient 1: 22-year-old Male with Refractory Hodgkin Lymphoma

- Medical Background: The patient, an IT student, was diagnosed with Hodgkin lymphoma and has undergone six cycles of first-line treatment. Despite this, his lymph node biopsy and imaging studies show refractory disease. Given his young age, he may be a candidate for autologous hematopoietic stem cell transplantation (AHSCT).
- Current Treatment Plan:
  - 1. Biopsy Site Care: Removal of three stitches from his left chest biopsy site.
  - 2. **Pre-AHSCT Therapy:** He will undergo two cycles of R-GDP salvage chemotherapy (rituximab, gemcitabine, cisplatin) over approximately eight to nine weeks. Each cycle lasts eight days, followed by a month of observation to assess response.
  - 3. **Peripheral Access:** A peripheral PICC line will be inserted for chemotherapy, as it is preferable over a catheter.
  - 4. **Post-Chemotherapy Evaluation:** PET scan after salvage chemotherapy to assess for remission.
  - 5. Oral Medications: Prednisolone, allopurinol, and lansoprazole.
- Learning Objectives:
  - 1. **First-Line Treatment for HL:** Familiarize myself with the typical first-line treatments for Hodgkin lymphoma.
  - 2. Role of Radiation Therapy in Refractory HL: Consider radiation therapy (RT) for local disease that doesn't respond to chemotherapy, weighing potential side effects like mediastinal inflammation and esophagitis.
  - 3. **AHSCT Process:** Understand the AHSCT procedure, including the necessity of a one-month hospital stay for transplant monitoring.
  - 4. Chemotherapy Side Effects and Management:
    - A. **General Side Effects:** Nausea, vomiting, and diarrhea, managed with antiemetics and symptomatic medications.

- B. **Mucositis and Ulcers:** Damage to rapidly dividing mucosal tissues; managed with mouthwashes.
- C. **Hematologic Toxicity:** Anemia, fatigue, bleeding (low platelets), and infection risk due to low WBCs.
- D. **Cisplatin-Specific Side Effects:** Risk of kidney damage and hearing abnormalities, necessitating hydration and monitoring for ototoxicity.
- 5. Oral Medication Functions and Precautions:
  - A. **Prednisolone:** Controls inflammation around tumor cells. Avoid use before biopsy to prevent inconclusive results.
  - B. Allopurinol: Helps clear byproducts from the breakdown of tumor cells.
  - C. **Lansoprazole:** A proton pump inhibitor given to prevent gastric ulcers due to steroid use.

#### Patient 2: 62-year-old Male with Stage IV Lymphoma (Spleen Involvement)

• Medical Background: The patient, in stage IV lymphoma with spleen involvement, reports low back pain. A PET scan is pending, and recent CT imaging shows no significant changes. His back pain could be due to injury (the patient is a golfer). Although he has not yet begun chemotherapy, bone marrow involvement may become a concern once treatment begins, which could lead to anemia and low blood cell counts.

#### • Current Treatment Plan:

- 1. **New Chemotherapy Regimen:** Cytarabine (high dose) + Rituximab, as it offers a higher remission rate compared to the traditional R-CHOP protocol.
- 2. **Infection Monitoring:** Body temperature monitoring is critical, as fever is often the first indication of infection in chemotherapy patients with neutropenia.
- Learning Objectives:
  - 1. **Understanding Drug-Specific Side Effects:** Study the side effects of cytarabine and rituximab.
  - 2. **Steroid Management:** Recognize the effectiveness of steroids in treating lymphoma but understand the increased risk of diabetes mellitus.
  - 3. **Fever Management in Chemotherapy Patients:** Avoid paracetamol as it may mask fever, the primary indicator of infection in neutropenic patients. If fever occurs, IV antibiotics should be administered within one hour.
  - 4. **Proactive Symptom Management:** Encourage regular use of supportive medications for chemotherapy side effects to prevent symptoms from escalating.

## **Reflections and Insights**

Today's cases highlighted the complexity of treating refractory and advanced-stage lymphoma. Key insights included the importance of thorough infection control in immunocompromised patients, careful symptom management, and understanding the potential complications and side effects of intensive chemotherapy regimens. The discussions also underscored the critical role of supportive care and detailed patient education, especially regarding the side effects and management strategies for chemotherapy and steroid use.

Learning about AHSCT and the considerations for refractory lymphoma treatment has enriched my understanding of hematologic oncology, particularly in how treatment plans are adapted for young, high-risk patients. Through this experience, I aim to deepen my clinical knowledge in managing lymphoma and improving patient quality of life during intensive treatment regimens.

#### **End of Report**

## **Learning Report**

Date: November 4 Department: MPN Clinic

## Patient Summaries and Learning Points

# Patient 1: Female with Primary Myelofibrosis (PMF), Post-Breast Cancer, and Leukemia

#### • Medical Background:

- 1. Patient with primary myelofibrosis, confirmed by JAK mutation, and a history of breast cancer (post-radiotherapy) and leukemia.
- 2. Chief complaints include fatigue and lightheadedness. Blood pressure was lower at this visit, and lab results showed hemoglobin at 6.2 and platelets at 11 (previously 35-40).

#### • Current Treatment Plan:

- 1. Momelotinib (JAK inhibitor) will be paused until platelet count reaches 70-80.
- 2. Scheduled for a blood transfusion and introduction of danazol to support blood count.
- Learning Points:
- 1. **Differentiating Primary from Secondary Myelofibrosis:** Primary myelofibrosis is confirmed by specific genetic mutations, such as JAK2 V617K, CALR, or MPL mutations, which distinguish it from secondary myelofibrosis caused by other underlying conditions. JAK mutations lead to continuous activation of the JAK2 protein, causing abnormal cell proliferation and fibrosis in the bone marrow.
- 2. **JAK Inhibitors in PMF:** JAK inhibitors, such as momelotinib, help control symptoms like splenomegaly and constitutional symptoms (e.g., weight loss, night sweats, fatigue) but do not reverse the underlying bone marrow fibrosis. Common side effects include a temporary drop in blood cell counts, which is closely monitored.
- 3. **Danazol for Blood Count Support:** Danazol is a mild androgen that supports platelet production, often with minimal side effects. Potential side effects include mild liver enzyme elevation, skin rash, and temporary hair loss. It is an option when other agents, like flutamide, pose a higher risk (e.g., clotting risk).
- 4. **Introduction to Momelotinib:** Momelotinib is a newer JAK inhibitor approved in 2024 in the UK. It offers another option in addition to ruxolitinib and fedratinib, commonly used for PMF management.
- 5. Distinguishing Spleen from Kidney Masses on Physical Exam:

- A. **Spleen:** Moves with respiration, has a notched edge, appears dull on percussion, and enlarges diagonally across the abdomen.
- B. **Kidney:** Does not move significantly with respiration, lacks a notched edge, often retains resonance (retroperitoneal position), and enlarges downward rather than obliquely.
- 6. **Reactive Thrombocytosis (Secondary Thrombocytosis):** This condition is often due to infections, inflammation, tissue damage, iron deficiency, malignancies, or recovery from thrombocytopenia. Differentiating it from primary thrombocytosis requires identifying the underlying cause through lab tests and imaging.
- 7. **Reactive Erythrocytosis (Secondary High Hemoglobin):** High hemoglobin due to secondary causes like chronic hypoxia, dehydration, or erythropoietin-secreting tumors needs to be differentiated from primary erythrocytosis. Dehydration, obesity, and stress can lead to pseudo-erythrocytosis without actual red blood cell overproduction.

#### Patient 2: Female with Essential Thrombocythemia (ET) and Breast Cancer

#### • Medical Background:

- 1. Diagnosed with ET and breast cancer. On hydroxycarbamide for cytoreduction.
- 2. Chief complaints: Leg cramps, numbness in fingers and toes.
- 3. Lab results: Stable platelet count (290) but decreased WBC (neutrophil 1.2).

## • Current Treatment Plan:

Discontinue interferon and hydroxycarbamide.

## • Learning Points:

- 1. **Thrombosis in ET and PV:** In ET and polycythemia vera (PV), thrombosis tends to be arterial, and smoking is a significant risk factor. Management often involves addressing lifestyle factors, such as smoking cessation, alongside medication.
- 2. **Hydroxycarbamide for Cytoreduction:** Commonly used in patients over 60 or with a history of clotting, hydroxycarbamide reduces platelet counts to prevent thrombosis. Monitoring blood counts is essential as over-suppression can lead to cytopenia.

## Patient 3: 79-Year-Old Male with Essential Thrombocythemia (ET)

## • Medical Background:

- 1. Longstanding ET managed with hydroxycarbamide; however, platelet counts remain elevated (580).
- 2. History of stroke, atrial fibrillation, high cholesterol, and migraine (currently on propranolol).
- 3. Chief complaints: Fatigue and weakness.

## • Current Treatment Plan:

Increase hydroxycarbamide dose and perform a bone marrow biopsy. Patient is positive for CALR mutation and may be eligible for a clinical trial involving bispecific antibodies or pegylated interferon-alpha.

#### • Learning Points:

- 1. **Macrolide Side Effects:** Includes gastrointestinal symptoms such as nausea, vomiting, abdominal pain, and diarrhea, which were reasons for stopping this treatment in the patient.
- 2. **Interferon Side Effects:** Interferon can cause skin rash and platelet decrease. Symptoms often resolve when treatment is paused or discontinued.

#### Patient 4: 36-Year-Old Male with Eosinophilia and Myocarditis

#### • Medical Background:

Recently diagnosed with eosinophilia and myocarditis, possibly due to vasculitis or autoimmune disease.

#### • Current Treatment Plan:

Initiated on steroids with consideration for methotrexate or cyclophosphamide to manage vasculitis if needed for long-term care.

- Learning Points:
- 1. **Eosinophilia and Organ Involvement:** Eosinophilia can lead to multi-organ damage, such as myocarditis, CNS symptoms, lung involvement, and skin manifestations. This patient requires treatment due to organ impact, with the goal of preventing further damage.

#### 2. Differential Diagnosis for Eosinophilia:

- A. **Infection:** Commonly due to parasitic infections.
- B. Allergy: Drug reactions, asthma, eczema.
- C. Autoimmune Diseases: EGPA, RA, PAN.
- D. Endocrine Disorders: Adrenal insufficiency.
- E. Neoplasm: Certain cancers, such as Hodgkin lymphoma.
- F. Atheroembolic Disease: Associated with cholesterol emboli.
- G. **Hypereosinophilic Syndrome (HES):** Multi-organ involvement, typically resistant to steroids.

#### Patient 5: Systemic Mastocytosis

#### • Medical Background:

Diagnosed with systemic mastocytosis, with stable platelet count (66), neutrophil (1.7), and tryptase levels (5.4). Currently stable without KIT inhibitor treatment.

• Current Treatment Plan:

Regular monitoring as condition remains stable.

- Learning Points:
- 1. **Mastocytosis Overview:** A rare disease where mast cells proliferate excessively, affecting various organs. Symptoms include allergic reactions, bone pain, GI issues, and in severe cases, anaphylactic shock.
- 2. Mastocytosis Subtypes:
  - A. Indolent (ISM): Slow progression with milder symptoms.
  - B. **Smoldering (SSM):** Higher mast cell burden.
  - C. Aggressive (ASM): Rapid progression with significant organ damage.
  - D. Mast Cell Leukemia (MCL): Severe and life-threatening.
- 3. **Diagnosis and Treatment:** Diagnosis often includes elevated serum tryptase, bone marrow biopsy, and KIT mutation analysis. Treatment ranges from antihistamines to tyrosine kinase inhibitors like midostaurin for advanced cases.

#### **Reflections and Insights**

This clinic session offered in-depth exposure to various aspects of MPN and related disorders, from managing complex myelofibrosis cases with JAK inhibitors to differentiating conditions with similar presentations (e.g., spleen vs. kidney masses). The experience underscored the need for a tailored approach when managing hematologic malignancies, taking into account both the genetic basis of diseases and the individual patient's tolerance to treatments. Understanding the nuances of medication management, such as side effects from interferon and hydroxycarbamide, highlighted the importance of balancing therapeutic benefits with potential adverse effects. Furthermore, learning to distinguish the underlying causes of eosinophilia and secondary erythrocytosis expanded my diagnostic skill set, reinforcing the value of comprehensive assessments in complex cases. Through this session, I gained a deeper appreciation for the multifaceted approaches required in hematologic care, where precision and patient-specific strategies are essential for effective, compassionate treatment.

#### **End of Report**

## **Learning Report**

Date: November 12 Department: Red Cell Clinic

## Patient Summaries and Learning Points

## Patient 1: 53-Year-Old Male with Sickle Cell Disease (SCD) and HbS-ß Thalassemia

#### • Medical Background:

Middle-aged African school teacher with a history of multiple annual pain crises typically managed at home. Recently, more severe episodes required hospital care. Past complications include Bell's palsy, a lung collapse (managed with exchange transfusion 15 years ago), occasional exertional dyspnea, and priapism during adolescence.

#### • Pain Management:

Effective pain relief is achieved with diamorphine, moderate relief with morphine, and minimal benefit from oxycodone. Current medications include folic acid, dehydrocodeine (used during crises), and erythromycin.

#### • Additional Health Issues:

Splenic atrophy identified on recent ultrasound, degenerative changes in the spine (advised to limit heavy lifting), past chest infection (resolved), and scheduled eye exams to assess blood vessels. Gallstones suspected but not confirmed in recent ultrasound.

#### • Current Plan:

Explore hydroxyurea or hydroxycarbamide for symptom control. Consider gene therapy or allogeneic bone marrow transplant in the future. Stronger pain management options may be considered if necessary, including fentanyl. Scheduled ophthalmology referral for regular eye monitoring.

#### • Learning Points:

HbS- $\beta$  thalassemia combines features of sickle cell disease and beta-thalassemia, causing anemia, pain crises, and potential complications like splenic sequestration and organ damage. Treatments include hydroxyurea, transfusions, and possibly bone marrow transplantation, depending on severity. HbS- $\beta^{0}$  thalassemia tends to be more severe than HbS- $\beta^{+}$ , which presents with milder symptoms.

#### Patient 2: Monk with Non-Transfusion-Dependent Beta-Thalassemia (NTDT)

#### • Medical Background:

Diagnosed with beta-thalassemia (NTDT) and currently on a Mitapivat drug trial aimed at boosting ATP in red cells, enhancing their longevity.

#### • Treatment Experience:

Patient reports reduced fatigue since beginning the drug trial over two years ago and attends a trial clinic every three months for monitoring.

#### • Complications:

Age-related chronic anemia, bone marrow expansion in atypical locations (spine, spleen, liver), iron overload, and potential gallstones (mainly fat-based).

#### Patient 3: 43-Year-Old Male with Secondary Polycythemia

#### • Medical Background:

Diagnosed with polycythemia related to a right-to-left shunt causing hypoxia. Current hemoglobin level is 191 g/L, managed primarily through phlebotomy.

#### • Management:

Phlebotomy continues as the primary treatment approach to reduce blood viscosity and minimize the risk of blood clots.

## • Learning Points:

Polycythemia can be primary (Polycythemia Vera, PV) or secondary (in response to hypoxia). PV, often caused by JAK2 mutations, leads to excessive red cell production and is treated with phlebotomy, low-dose aspirin, and sometimes hydroxyurea. Secondary polycythemia is managed by treating the underlying hypoxia. Diagnosis involves blood tests (CBC, EPO level), genetic testing (JAK2 mutation), and sometimes imaging to rule out secondary causes.

#### Patient 4: Young Male with Thalassemia Intermedia

#### • Medical Background:

Diagnosed with thalassemia intermedia, a moderate form of thalassemia, and referred to rheumatology for connective tissue disease (CTD) concerns.

#### • Learning Points:

Thalassemia intermedia is characterized by milder anemia, fatigue, and possible bone deformities due to marrow expansion. Treatment may include occasional transfusions, folic acid, iron chelation (if needed), and sometimes splenectomy. Complications include iron

overload, bone deformities, and increased thrombosis risk. Regular monitoring of iron levels and bone health is crucial to manage long-term outcomes.

#### **Reflections and Insights**

Today's cases provided a deeper understanding of complex hemoglobinopathies and the tailored approaches to managing chronic conditions like SCD, NTDT, and polycythemia. Key insights included the importance of individualized pain management, the role of novel therapies like Mitapivat, and the challenges of managing secondary effects like iron overload and bone complications in thalassemia. These cases highlighted the value of integrated care plans and regular monitoring in preventing long-term complications. Through these experiences, I aim to enhance my knowledge in managing chronic hematologic disorders and refine my approach to providing comprehensive patient care.

#### **End of Report**