c/o Thalassaemia Association of Hong Kong
Room 201-203, Nurses' Quarters Block B1
Queen Mary Hospital
102 Pokfulam Road
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14 May 2008

Hon LI Kwok-ying, MH, JP
Chairman, Health Services Panel
Legislative Council
Legislative Council Building
8 Jackson Road
Central, Hong Kong

By email: panel_hs@legco.gov.hk

Request for Better Iron Removal Therapy from the Hospital Authority

Dear Mr. Li

We write to ask for the Health Services Panel to help liaise with the Hospital Authority for a better iron removal therapy for Thalassaemia patients in Hong Kong at the earliest convenience.

Severe Thalassaemia patients require blood transfusions every two to four weeks and as a consequence lead to excessive iron deposition in our body. Excessive iron in the body damages the heart and liver, and the consequences are fatal unless the iron is removed. Therefore, patients also need iron-removal therapy called deferoxamine (DFO), which is a subcutaneous infusion that takes as long as twelve hours or more for each session.

Currently, there are three types of iron removal therapy available in Hong Kong:
1. Traditional iron subcutaneous removal infusion deferoxamine (DFO)
2. Traditional oral iron removal therapy deferiprone (DFP)
3. New generation oral iron removal therapy deferasirox

Traditional iron subcutaneous removal infusion deferoxamine (DFO)
Currently, public hospitals are providing DFO to Thalassaemia patients for removing excessive iron from the body.

DFO is a subcutaneous infusion, taken through a needle (often at abdominal) and each session takes twelve hours or longer, five to seven sessions per week. The drug itself is free of charge provided by HA, but, patients still have to fund the treatment pump (roughly about HKD7000 each) and disposable syringes (roughly about HKD750 each month).

This time-consuming regimen poses several challenges:

- **Bone Deformity:** Many patients are suffering from bone deformity, including short height, broad shoulders, U-shape legs and protruding breast bones due to the side effects of DFO. Some of us have to undergo surgery to rectify these medical conditions. In addition, the deformity causes us an odd appearance making it difficult to find work.

- **Scarring, Swelling and Hardening of the Skin:** Over the years, the painful infusion treatment causes irreversible scarring and hardening of the skin, as well as persistent swelling at the injection site. Due to this side effect, many of us have to intermittently skip infusion, adversely impacting the outcome of the treatment, leading to further health issues and higher health care costs.

- **An Inability in Staying Productive:** The infusion requires binding a treatment pump, which is like a box (sized 5 cm x 16 cm), that not only limits us from resting fully, but also limits our productivity at work or school in the daytime.

- **A Limitation on our Social Lives:** The daily twelve-hour long infusion means that we can never participate in nighttime activities and absolutely must leave work by 6pm, a feat that is difficult to achieve in view of the prolonged working hours in this city. The negative impact on the ability to engage in social interaction often has a corresponding negative impact on the mental and psychological well being of the patients. This affects all aspects of life for the thalassaemia patients, including self-confidence, productivity, and even the will to live. Worldwide, many thalassaemia patients have chosen death rather than comply with the painful subcutaneous nightly injection, which in the eyes of many patients, makes life not worth living. Patients in Hong Kong are no exception and non-compliance is the single most important factor leading to the premature deaths of patients. This is the saddest fact about treatment with DFO. Access to an oral, easy to use, iron removal drug, is essential for patients to lead normal lives.

Traditional oral iron removal therapy deferiprone (DFP)

The Hospital Authority does provide a thrice-daily oral medication, L1 (deferiprone) DFP, as an alternative to DFO. However, only some patients with medical indications are given L1 free of charge. Moreover, DFP has a plethora of side effects, rendering it unsuitable for many patients.

Some DFP patients experienced a drastic reduction of white blood cells and a weakening of the immune system, resulting in symptoms such as serious mouth ulcers and a persistent fever, and even death in some cases. Many patients also experience severe joint pain, resulting in discontinuation of the drug. Some have required hospitalization in the isolation ward for two weeks, due to a risk of severe life threatening infections, which adds significant medical cost. These side effects are not isolated incidents and we widely recognized in the medical community. In 2006, the National Health Service in the United Kingdom published a letter to doctors worldwide to ask for weekly monitoring of the white blood cell level for patients treated with DFP. Not only does the weekly monitoring pose extra medical cost, but many of us are also struggling with the weekly monitoring schedule and normal work and school life. While DFP is effective in some patients, its side effects prohibit its use in far too many patients, thereby eliminating it as an option for the majority of Hong Kong thalassaemia patients.

New generation oral iron removal therapy

A new generation oral iron removal therapy, deferasirox (also known as “Exjade), is approved by the Hong Kong Department of Health in 2006 for treating patients aged 2 and above who have iron overload as a result of blood transfusion.

This new generation oral iron removal therapy is a once-daily therapy and can be dissolved in drinks. As such, it overcomes challenges posed by traditional subcutaneous infusion and helps patients comply with doctors’ instruction. The side effects of this deferasirox are transient with mild skin rashes, stomach upsets and stomach aches. These side effects are normally not serious enough to cause any reduction in use of the drug, and typically vanish after some weeks of use.

In 2007, the Hospital Authority enlisted this treatment as a “self-financed item” under the drug formulary, meaning patients have to pay the full cost of treatment. The average treatment cost per patient per month

is roughly around HKD 18,000. Because no one iron removal drug can meet the needs of all patients, thalassaemia experts recommend that all three drugs be made available, so that patients can choose the drug that best suits them. Every attempt needs to be made to guarantee that all patients have access to the medicines that can give them equal access to normal lives. We are asking that our lives not be weighed against the cost of treatment.

Our Request – Reimbursement of New Generation Oral Iron Removal Therapy

We hope the Hospital Authority can reimburse the new generation oral iron removal therapy, deferasirox.

Although deferasirox seems to be more expensive than traditional therapies, it saves overall medical costs and sufferings due to side effects such as treatment of neutropenia, surgery for deformed bones and heart failure due to iron removal. In addition, the new therapy adds productivity as it enables us to lead a normal life and continue to contribute to society. Attached please find the recent SCMP's interview about patients who are receiving new therapies and are leading a much more productive life.

Since the government has successfully introduced prenatal screening in the 1980s, there are only one to two new cases of Thalassaemia Major. The total number of Thalassaemia Major patients, which currently is 379, is now capped and will continue to decline as patients pass away.

We wish the Hospital Authority to follow the steps of governments of neighboring countries such as Macau, Taiwan, South Korea and Australia, which have already agreed to reimburse deferasirox. We hope that you will see that a positive response from your panel will help give us an equal opportunity to both life itself and also the quality of life that we are allowed to lead. As citizens, we should have every expectation that we will be given the same opportunity to live and fulfill our potentials as every other citizen.

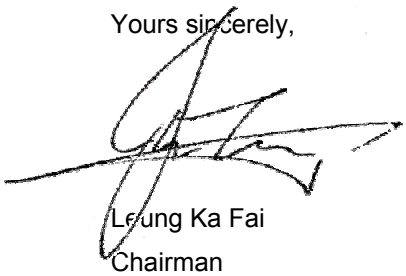
We, the thalassaemia patients of Hong Kong, appreciate the challenges that the HA is facing and are prepared to work hand-in-hand with the Authority to overcome challenges. We had sent a written request to Mr. Shane Solomon, CEO of Hospital Authority for a discussion on 23 April 2008 but until now, we have not received any response.

We would be grateful if this issue could be discussed at the health service panel as soon as possible so that Thalassaemia patients in Hong Kong can have access to better treatments sooner.

Please don't hesitate to contact Jessis Ng, Coordinator, Children's Thalassaemia Foundation or Mandy Yuen, Officer, Thalassaemia Association of Hong Kong at 2523 5400 if you have any questions.

We are looking forward to your favourable feedback.

Yours sincerely,



Leung Ka Fai
Chairman
Thalassaemia Treatment Patient Concerned Group

c.c: All health services panel members.

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Sufferers of a genetic blood disorder are finding the strength to pursue their dreams, writes **Charmaine Carvalho**

Alive and kicking

Under the stage lights, Chen Sze-kwan from hip hop group Fama is pumping it up, raising the excitement level at a concert in Wan Chai. It's hard to imagine but the 24-year-old has had to have a blood transfusion every four weeks since he was a baby. C Kwan, as he is better known, suffers from thalassaemia, a genetic blood disorder that inhibits haemoglobin production.

Kwan remains upbeat as he raps about the ups and downs of life in Hong Kong. Diagnosed when he was four months old, he is used to the treatment and says he grew up like any other child – he was even on his school table tennis team.

"It didn't affect my extracurricular activities at all," he says. "Once a month I had to take a day off to go to the hospital, and I quite liked having the holiday."

Despite Kwan's nonchalance, it couldn't have been easy to cope with the treatment. Repeated transfusions cause iron to be accumulated in the body and the excess must be removed regularly through an injection of drugs to the stomach that lasts up to 12 hours.

"This kind of prolonged injection through the night causes a lot of pain and discomfort," says Li Chi-kong, a doctor who helps run the Children's Thalassaemia Foundation. "When they hit adolescence, especially, patients can be less co-operative but if they do not receive the treatment regularly, problems start. They may develop heart disease, liver problems, diabetes and other complications."

The incidence of thalassaemia is relatively high in Hong Kong. One in 11 people, or 8.5 per cent of the population, carries the gene and can pass the illness to their children. That's more than three times the rate of 2.54 per cent of people in neighbouring Guangdong province. The incidence in Shenzhen is even lower at 1.99 per cent, which some researchers attribute to a large influx of people from northern China, who are less likely to have the gene.

Thalassaemia is the most common genetic disorder in the world, Li says, and affects people living within a belt stretching from the Mediterranean to southern China. Although it isn't clear why they are more prone to thalassaemia, one theory is the blood composition makes them more resistant to malaria, which is common in those regions.

In its severe form, the inability to produce sufficient haemoglobin causes long-term anaemia that may lead to an enlarged liver and spleen, bone deformities and inhibited growth.

Carriers are asymptomatic and do not require treatment. But since there is a 25 per cent chance of passing the severe form of the disease to their children, pregnant women in Hong Kong are tested to prevent new cases developing.

The foundation, which seeks to raise awareness about the illness, is marking International Thalassaemia Day today by organising a Jeans for Genes campaign. Office workers and students get the chance to wear jeans for the day by making a small donation to aid research and treatment for poorer sufferers.

Lack of public awareness can create difficulties for many sufferers. "People stare at me on the street but my parents tell me it's because they don't know better," says 19-year-old Angel Yeung Sheung-yu, whose physique was stunted by the illness.

That also makes getting a job tougher. "Some employers thought I had a mental illness; others worried that I would take too much leave," she says. Now working as a receptionist, Yeung says she enjoys the job but can get very tired just before she is due for a transfusion because her haemoglobin count is low.

Angela Chung Hei-tung, a 15-year-old student, is helping to educate people about her illness. Encouraged by her mother, Susanna Chung Ip Pik-san, she has appeared on TV since she was four to talk about living with thalassaemia.

As a toddler, Angela found it hard to explain to friends that the illness wasn't contagious but her public appearances helped. "My school principal saw me on TV and explained to the school about [the illness] at assembly the next day. After that people understood.

"I can't do very physical sports like volleyball, which I enjoy watching, but I have my best friends and teachers and parents who love me so I don't think I've missed much," she says.

Sufferers must also avoid certain iron-rich foods such as beef, apples and chocolate, although Angela admits she sneaks the odd sweet when her mother isn't looking.

Chung is proud of her daughter. "There are some parents who hide that their child has thalassaemia, even from their own relatives," she says. "But I feel this is harmful for the child – she needs to accept her illness."

Angela thinks it's important to let more people know about the disorder and coping with lengthy iron-removal treatments. She still winces at the thought: "The needle is quite thick, so it hurts," she says. Yeung says she has become used to the all-night drug regimen, although it restricts her activities. "I can't stay out longer than 9pm so I can never experience Halloween," she says. "Going camping means I must make up for skipping the treatment by undergoing a whole-day injection, which is not healthy."

Oral medications were introduced in 2000 as an alternative to painful injections. However, patients don't always respond well to the drugs, which can can cause joint pain and a dangerous reduction in the white cell count. Another oral medicine, which suits patients better, became available in 2006, but the high cost – up to HK$200,000 a year for an adult – puts it out of reach of many sufferers.

Fortunately, Angela isn't among them. Thanks to the new medication, which is taken only once a day, she can indulge her passion for acting. "Previously, I had to rush home from school to get ready to take the injection so I couldn't stay on for the drama," says the student, who has won the award for outstanding actress in her school drama festival for the past two years.

The new drugs have also allowed Kwan to lead a relatively unfettered life, including the many late-night performances that are part of pursuing a pop career.

"Most people in the industry know about [the illness] and it hasn't been a problem at all," he says. "I have a busy schedule with writing, recording, performing and attending events, so I feel quite tired sometimes. But I can't say whether that's because I have thalassaemia.

"There are those who see themselves as unfortunate but we are lucky in many ways because we can get advanced treatment in Hong Kong," he says. "We can achieve our dreams despite this illness if we're optimistic."

Angela Chung and Adam Ho suffer from thalassaemia, a blood disorder that causes fatigue and can lead to complications if not treated correctly

We can achieve our dreams despite this illness if we're optimistic

Chen Sze-kwan (above)

For more information, visit thalassaemia.org.hk or call 29863311

爭取將新一代口服除鐵藥Deferasirox 列作第一線除鐵藥物

背景：

醫院管理局藥物名冊

根據現行機制，藥物分爲三個類別：

- 通用藥物
- 專用藥物
- 自費藥物

「自費藥物」的定義是：

- 證實療效顯著但卻極度昂貴的藥物，超出醫管局一般資助服務的範圍
- 僅經初步醫療驗證的藥物
- 與其他替代藥物相比，僅具邊緣效益、療效略佳但成本卻明顯高昂的藥物；及
- 生活方式藥物

在推出藥物名冊時，雖然醫管局聲稱病人不會因此機制而得不到適當治療，但自名冊推出後，很多先進、有效但昂貴的藥物都被納入病人自費類別。相反，舊式、不安全、有明顯副作用的藥物卻仍保留在通用或專用藥物類別。

地中海貧血症 (地貧)

地貧是全球最常見的基因遺傳疾病。重型地貧患者因嚴重缺乏正常血紅蛋白，以致身體無法自行製造紅血球，須終身接受輸血。現時，全港約有 374 名重型地貧患者，包括成年人、青少年及兒童，他們全部需要長期輸血。[i]

其實早於 80 年代初，香港已推行產前檢查，自此每年新增的地貧個案只有數個，預計未來地貧病人數目只會有限度增長，而醫管局須在地貧治療方面投放的資源，亦不會大幅增加。

重型地貧病人必須每四星期到醫院接受輸血，以維持生命。他們每星

期亦要有 5 至 7 晚進行 8 至 12 小時的皮下除鐵注射，以防止鐵質積聚。

若體內積聚過量鐵質，可能會令多個器官受損，導致各類嚴重疾病，例如心臟衰竭、肝炎、肝硬化、腎衰竭等等。

通用藥物 — 皮下注射除鐵劑 (DFO)

現時，公立醫院免費爲地貧病人提供的除鐵藥，就是傳統皮下注射除鐵劑(DFO)，通常注射在肚皮，每次需要 10 至 12 小時，每星期 5 至 7 次。由於注射除鐵藥需時，故病人通常在睡眠時進行注射。注射時病人必須把一個體積約 5 厘米 cm x 16 厘米的打針機繫在身上。因此長久以來，地貧病人的睡眠質素往往欠佳。

經年累月的皮下注射對病人構成很大影響，當中包括：

- **骨枯、骨質疏鬆：**傳統的皮下注射除鐵藥在清除體內多餘鐵質的同時，亦一併除去有助成長及骨骼生長的元素，因而病人的身高及骨骼都會受到影響。不少病人的個子都較矮小，亦有病人在青春期已出現骨枯或骨質疏鬆情況。爲減輕副作用，很多病人經常會暫停治療。

- **傷疤、硬皮及注射位置長期腫脹** — 經年累月的注射令病人早於青春期時，已經出現傷疤、硬皮及注射位置長期發炎、紅腫等問題。很多病人都難以在身上找到合適部位以作注射；亦有病人感到注射過程太痛苦，寧願冒著鐵質積聚過多至心臟或肝臟衰竭的風險，選擇自行終止或間歇性停止注射。

- **不能安睡、影響學習與工作能力** — 打針機的體積爲 5 厘米 x 16 厘米，注射時病人需將其繫在身上，不能隨意翻身，長期影響睡眠質素。不少病友因睡眠質素惡劣，休息不足，影響日間工作及學習能力。

- **日常工作、社交生活受嚴重影響** — 由於每次注射需要 10 至 12 小時，地貧患者往往難以依時上班/上學，亦無法超時工作。然而，香港是全球工時最長的城市之一，不少地貧患者既要爲口奔馳，又渴望獲得足夠治療，感到進退兩難。

專用藥物 — 傳統口服除鐵藥 deferiprone（DFP）

根據現時的藥物名冊，DFP 被列爲「專用藥物」類別，可減少病人注射次數及減輕傳統皮下注射劑的副作用。

醫生多選用 DFP 作單一療法或配以 DFO 作混合治療，旨在減少病人注射次數及減輕傳統皮下注射劑的副作用。不論是單一或混合療法，兩者均能有效改善病人在心臟的鐵質積聚。

然而， DFP 可能令病人出現嗜中性白血球減少症或粒性白血球缺乏症[1]。另外，DFP 只核准治療 10 歲或以上病人。至於年紀較小的病人，仍只可使用 DFO。

由於這兩種嚴重病症的初期並無任何症狀，故病人在服藥時期間，必須每星期到醫院驗血，加重醫療負擔。如不及時治理，這兩種病症可導致嚴重症狀，例如口腔潰瘍、持續發燒，甚至因白血球過低而失去免疫力，引起致命的併發症。在治理時，醫生可能需要安排情況嚴重的病人入住深切治療病房，以減低感染致命病毒或細菌的機會，大大加重醫療開支的重擔。

出現白血球過低的情況並非單一個案，有關藥商早於 2006 年知會全球醫生，指藥物可能引致上述兩種嚴重情況，並要求全球醫生在處方此藥予病人時，必須規定病人每星期須抽血檢查白血球的水平。

自費藥物 — 新一代口服除鐵藥 Deferasirox

新一代的口服藥 Deferasirox，在 2006 年 8 月獲香港衛生署核准使用作治療因輸血而引致鐵質過多的兩歲或以上地貧病人。

此新一代的口服藥物每日只須服用一次，並可溶於飲品內，故能大大改善病人使用傳統除鐵注射的困難，令病人更容易遵循醫生指示。此藥物的副作用較小，例如短暫性及程度輕微的皮疹、腸胃不適及腹痛。

在 2007 年初，醫管局將此藥納入藥物名冊內的「病人自費藥物」類別，病人須自行負擔藥費。

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[1] Thalassaemia International Federation, Guidelines for the Clinical Management of Thalassaemia, 2nd edition, P.50

現在，全球有接近 20 個國家是由政府全數承擔 Deferasirox 藥費，包括鄰近的澳門(附錄是澳門醫生信)、台灣、南韓及澳洲。

地貧病友的立場

- 生命是寶貴的，不能以「合理使用公共醫療資源」爲理由而犧牲少數地貧病友。
- 藥物講求療效，只要對病人有幫助的藥物，無論病人數目多少，一經醫生處方，就不應有人因經濟問題而得不到治療的機會。
- 長期病患者應與政府共同解決醫療開支問題。

地貧病友的訴求

- 醫管局將新一代的除鐵口服藥 Deferasirox 納入通用藥物類別，免費爲病人提供。
- 儘速向地貧病友提供 Deferasirox，令他們不用再擔心會出現嚴重而致命的副作用。
- 醫管局與病人洽談將 Deferasirox 納入通用藥物類別的訴求。

地中海貧血病 - 藥物關注小組
二零零八年五月七日

[i] http://www.thalassaemia.org.hk, 2006