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Little People, Big Steps

Editorial Board

Editor-in-chief:

Prof Danny Chan

Editors:

Ms Serene Chu, Dr Vivian Tam,
Ms Miranda Chiu & Ms Shirley Cheng

Art Director:

Dr Wilson Chan



HEADLINE STORY:

“Little People Care Alliance” under the Knowledge Exchange Program at The University of Hong Kong

The Little People Care Alliance is an initiative that began in July 2014 under the Knowledge Exchange Project steered by the Department of Biochemistry at The University of Hong Kong. In collaboration with LPHK and St John's College HKU, Little People Care Alliance aimed to increase public awareness on the challenges faced by Little People and facilitate as a platform for discussion between affected families, the education sector, and policy makers so as to promote understanding and acceptance of patients with rare bone diseases.

Symposium “I’m Little but Not Less: Have I a place in your School” on 25 April 2015

The symposium aimed to raise awareness and promote an inclusive learning environment for Little People. We were honoured to have Dr York Chow, Chairman of Equal Opportunities, and Professor Danny Chan, Assistant Dean of Li Ka Shing Faculty of Medicine, HKU, to deliver enlightening opening remarks on the importance of equal education opportunities for Little People. Medical specialists, a family therapist, patients and their families also shared Little People's medical needs and the challenges they face in schools, including bullying. Principals and teachers from two schools also shared their experience in including Little People in their schools, which demonstrated how an accepting school environment can help Little People strive and excel. In the Q&A session, audience including educators, social workers, nurses and lawyers had fruitful discussions with the

speakers on issues of equal education opportunities and support for Little People.

Knowledge Exchange Booklet on rare bone diseases to be published in September 2015

This book is the first book in town that features the lives of Little People. It is a compilation of articles contributed by

medical practitioners and students as well as parents, interweaving heartening stories of both affected children and grown-ups. We hope that the 60-page book can give guidance to patients and their families, and raise public awareness on the obstacles faced by Little People every day. The book will be disseminated to schools, public libraries, hospitals, companies and relevant government departments in September 2015.

“Big Brother Big Sister Mentorship Programme 2014/15

In this programme, about 20 undergraduate students from HKU paired up with our LPHK families to participate in a series of activities such as Cupcake Fun Day, Ocean Park Visit and Chinese New Year Party.

Vision Tree on International Rare Disease Day on 28 February 2015

Similar to last year, we held an annual New Year Party with members on International Rare Disease Day. With the presence of our honourable advisers, Prof John Leong, Chairman of Hospital Authority and Mr Bernard Chan, Executive Council member, a vision tree was put up by LPHK families and Big Brothers & Big Sisters from HKU. The tree symbolised our hope for wider acceptance and support from the society such that Little People can grow and develop their full potential.





Medical UPDATE

Drug development: from the laboratory to commercial sales

- Screening of thousands of potential drugs in the laboratory with cell lines and identification of the best drugs for further testing
- Assess pre-screened drugs and dosages in animal studies to determine their effect
- Potential modification of drugs to enhance their activity and pre-clinical testing in animal studies
- Testing in non-human primates if applicable
 - Phase 1 clinical trial (test safety and dosage in patient subjects)
 - Phase 2 clinical trial (after safety and efficacy has been shown in phase 1 study)
 - Phase 3 clinical trial
- Approval by local authorities and commercial sales of drug

What are the Potential Treatments for Achondroplasia?

It is commonly known that there is currently no “cure” for achondroplasia. However, two potential drugs may bring hope for achondroplasia patients in the future.

VOSORITIDE

What is Vosoritide?

Also known as BMN 111, it is a drug developed by Biomarin Pharmaceutical which acts to promote bone growth.

How can this drug help patients with achondroplasia?

In a 6-month phase 2 clinical trial with young patients (where the growth plates are not yet fused), children with higher doses of the drug had up to 50% faster growth increase in comparison to their normal growth rate.

Is this drug under clinical trial?

A current follow-up 18 month study is being carried out on the patients from the phase 2 trial, with all patients on the higher dosage of the drug. A phase 3 trial is being planned in the future to also include children under the age of 5.

Are there side effects?

The phase 2 study reported some mild side effects including injection site reactions, headache, hypotension, back pain and cough. It is unknown if long term use can sustain growth.

Further reading:

<http://investors.bmrn.com/>

Nature. 2014 Sep 25;513(7519):507-11

STATIN

What is Statin?

Statin is a drug currently used to treat high cholesterol levels.

How can this drug help patients with achondroplasia?

This is a new discovery in a drug screen using cells from patients with achondroplasia. Statin stimulated production of better quality cartilage in patient cells. In mouse models with achondroplasia, statin can increase bone length to some extent.

Is this drug under clinical trial?

There is no current clinical trial for the use of statin in children with achondroplasia. A phase 1 clinical trial is being considered to determine the efficacy and dosage for safe use in children, as the cholesterol-lowering effects of statin could affect their development (cholesterol is necessary for building cell membranes and brain development). There is a suggestion that the modification of statin could potentially promote bone growth but without any effect on cholesterol levels. Active research is in progress to study its potential for clinical application.

Are there side effects?

Some reported side effects of statins in adults include: increased liver enzyme concentrations, increased risk of diabetes, muscle problems, cognitive loss, and neuropathy.



LPHK NEWS

Education, Policy & Public Awareness



OCT2014 Education and public awareness

Under the "Big Brother & Big Sister Mentorship Programme 2014/15", a Cupcake Fun Day was organised to promote Dwarfism Awareness Month.



NOV2014 | Education & Public Awareness

Vice-Chairman Prof Danny Chan, Executive Committee member Dr Brian Chung and Chairperson Ms Serene Chu delivered a 2-hour lecture on "Rare Skeletal Disease" to registered nurses and students from the Division of Nursing and Health Studies at the Open University of Hong Kong.



DEC 2014 | Education

Executive Committee members Dr Michael To, Prof Danny Chan, Mr Dennis Fong and Ms Serene Chu attended and spoke at the rare bone disease medical seminar cum patient gathering held at HKU-Shenzhen Hospital.



NOV2014 | Public Awareness

Chairperson Ms Serene Chu and Executive Committee member Mr Dennis Fong attended a programme entitled "Share my songs" on Commercial Radio Hong Kong to share their personal experience of having a child with dwarfism and the mission of Little People of Hong Kong Foundation.



FEB2015 | Education & Public Awareness

A Chinese New Year gathering to promote Rare Disease Day were organised as one of the activities under the "Big Brother & Big Sister Mentorship Programme 2014/15".



MAR2015/Education

Under the "Little People Care Alliance" Knowledge Exchange Project, a High Table Talk entitled "A long and winding road in dignity" was held.



APR2015 | Education & Public Awareness

Under the "Little People Care Alliance" Knowledge Exchange Project, a symposium entitled "I'm Little but Not Less: Have I a place in your school" were held.

Family & Patient Support



NOV2014 | Ocean Park Visit

An Ocean Park Visit was organised under the "Big Brother Big Sister Mentorship Programme 2014/15". Seven families and 13 HKU volunteers spent a joyful day at the Shark Mystique, Arctic Adventure, Giant Panda House and other attractions.

JUL2015 | "Music for everyone" Concert

LPHK members were invited by Music Children Foundation to attend the "Music for Everyone" Concert.



JUL2015 | Pottery Class

Ernst & Young Helping Hands and LPHK co-organised a pottery class for LPHK members.

A “Little Giant” on the badminton court



An energetic and active young man who always has a heart-warming grin on his face, Tim works at an IT department of a financial company during the day – just like any one of us. But at night, he transforms into a superstar on the badminton court! Tim is a representative of the Hong Kong badminton team short stature group, and just recently returned from an international badminton competition in the United Kingdom.

GREAT
PEOPLE,
GREAT
STORY

“No doubt there is greater support for patient groups in countries other than Hong Kong”, Tim expressed when meeting other overseas sports players with skeletal dysplasia. He found that the scale of overseas patient groups was larger than that of Hong Kong due to larger populations in other countries, which contributed to a larger pool of people with skeletal dysplasia. In addition, overseas patient groups had more support and interest groups to cater for a diverse number of activities ranging from sports teams for basketball, swimming and badminton, to study groups that assist with academic needs of younger patients, and other social activities such as hiking and dating.

When asked about LPHK, Tim agrees that “LPHK in Hong Kong is doing very well given that it is only at its beginning stage.” He compares the local and overseas patient groups and believes there are many opportunities for LPHK to explore and expand on.



UPCOMING EVENT

HKU x LPHK Knowledge
Exchange Project
Public Talk “Genetic Bone
Diseases”

2015/9/12 (Sat) 10:00 a.m
Hong Kong Central Library (Lecture Theatre)
Speakers:

Dr. CHUNG Hon-yin Brian
(Department of Paediatrics & Adolescent Medicine, HKU
Li Ka Shing Faculty of Medicine)
Dr. TO Kai-tsun Michael
(Department of Orthopaedics and Traumatology, HKU Li
Ka Shing Faculty of Medicine)



JOIN US!

If you would like to become a LPHK friend or volunteer, please visit <http://lphk.org/> to download the application form. Email your completed form to info@lphk.org or post it to Little People of Hong Kong, 2/F, Third Wing, St John's College, 82 Pokfulam Road, Hong Kong.

DONATIONS

You can also support us by donating to the Little People of Hong Kong.

By Cheque

Please make a crossed cheque payable to "Little People of Hong Kong Foundation Limited" and send to The Little People of Hong Kong Head Office, 2/F, Third Wing, St John's College, 82 Pokfulam Road, Hong Kong.

Please send the crossed cheque along with your name, address and contact email or telephone number to the foundation.

By Direct Bank Deposit

You can deposit your donation into our account - Bank of East Asia: 015-514-68-013177

Please send the deposit slip along with your name, address and contact email or telephone number to the foundation by fax at +852-28175624, by email to info@lphk.org or by post (address as above).

An official receipt will be issued for donations of HK\$100 or above.

