fd-uk

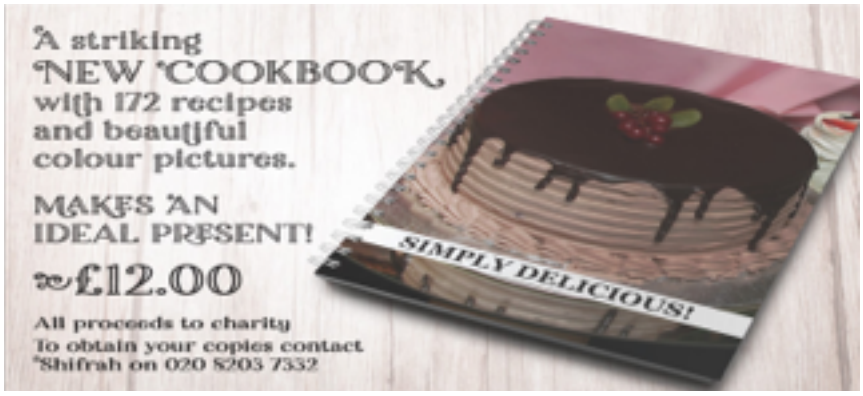
Buy our Newly Released Cook Book | Join Team FD-UK Maccabi Fun Run 2020

Welcome...

to our Winter 2019 newsletter. You will find information our new cook book now available to buy and other fundraising activities for next year. We also feature a special on the rare disease summit conference and our lifetime volunteer nomination. We would like to thank you for your support and for reading this, and being part of our FD-UK community. Together we can make a difference! within you all a bright and happy Chanukah!
Love from the FD-UK Committee X

FD-UK Nominated Bryan King for a Lifetime Achievement in Volunteering Award

Bryan King, is a Trustee and co-founder FD-UK and he has held key positions for over 40 years. FD-UK have nominate Bryan for JVN lifetime volunteering award. Winners will be announced December 2019.



Cook Book: All proceeds to FD-UK

The FD cook book is now available, get your copies for Chanukah gifts now in outlets near you! Stockists include Kosher Kingdom & Gorgeous Gifts (Golders Green) Flax's (Bushey); Luv-It, Noshers, Chabad (Radlett); and Be Kosher (Edgware). You can also buy it direct from us. Call Shifra 0208 203 7332 or Please email reply@Fd-UK.org

Rare Disease Summit Sept 2019

Our FD-UK Vice Chair Dr Shara Cohen attended the rare disease summit, 'patients as partners' a forum at Cambridge University.

This annual event is a joint meeting between UK patients, scientists and pharma who are involved in all aspects of the rare diseases.

The current situation regarding therapies is that only 5% of rare diseases have any sort of therapy at all. Only 10-15 orphan drugs are approved each year. The pace for developing a drug is too slow for people

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Up and Coming Events 2020:

FD-UK Day, May 2020

Put a date in the diary for next year xx May for FD sufferers, families and friends in Wolfson Court in Golders Green.

Save the date for next years Annual Supper Quiz

Join us for our annual supper quiz which will take place next November 2020 from at Hasmonean High School for Boys, Holders Hill Road NW4 1NA.

FD-UK Fun Run Join us next year: 5th July 2020

Would you like to join our FD-UK Team on the Maccabi Community Fun Run next year, you can walk, hop, run or roll with us on 5th July 2020, Allianz park. Do the 1K, 5K, 10K or Tri-Run or the colour run. Please email: reply@Fd-UK.org

who currently have a rare disease. Pharma, science and patient groups are all committed to finding cures quicker, faster and cheaper and they need to work together to get this. There are 7000 rare disease charities/patient groups that need to work together as they have a common goal. There was talk of starting a funding group to support patient groups (like FD-UK) to understand how to access ways to get research for their disease. Big Pharma say that small patient groups should not be scared of asking them for help.

The only current ways to get around the lack of drugs for rare diseases are:
1) Drug repurposing
2) Be better informed of label use
3) Find diseases with similar symptoms to crossover research

It is the patient community which holds the responsibility to push the research. Funding is not the only criteria needed for rare disease research. Collaborations and connecting different partners like patients and researchers is also very important.

There are 12 big Pharma companies (including Pfizer and GSK) who work together with scientists / universities to share data to assess disease progression and treatment. The aim is to reduce the time it takes to get treatments to the patients. Acceleration of research 'from bench to bedside' seemed to be the theme of the day. This is directly relevant for people with FD.

There is also a big effort to create hubs so that every person with specific rare diseases can get access to trials. Nurses, statisticians and everything that is needed is paid for by the hub to support pharma to enable patients to go in trials, as in the past people have been turned away from clinical trials run by pharma due to lack of funds.

There was also a lot of discussion around setting up a social fund for diseases that don't need drug development but need tech development or respite.

The Big Pharma who were there (AstraZeneca mainly) said that there is a loophole in drug costs where some (smaller) pharma will increase costs of drugs ridiculously and pressure on local MPs to increase the expensive drug supply seems to be the way forward.

Examples of what is happening with various rare diseases
Duchenne UK have repurposed tamoxifen (a drug I personally used for my cancer treatment) to treat symptoms of Duchenne. They think supply will be affected by Brexit, but have set up an

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international hub to overcome this.

The International Gauchet Alliance makes sure that no one intentionality is alone and empowers patients globally who cannot get treatment locally. They are developing wearable technology to assess diseases impact so that trials can properly assess/validate drug efficiency outcome.

Ring20 is a patient support group for ring chromosome 20 syndrome (less than 50 patients worldwide). Their emphasis is 'share if you are rare' and they encourage the ultra rare diseases to get together to share experiences and help with research together. This is very relevant to FD-UK which support the ultra rare disease FD. My opinion is that we should start to call FD an ultra rare disease as will have significance within the science community.

By being part of other groups we can
1) Raise the charity profile
2) Increase awareness
3) Increase information flow and education
4) Help with funding
5) Partner with others for research
6) Raise the patient voice

The overall message was the rare diseases need to work as a community. Pharma, scientists and patients need to work together and the rare diseases and ultra rare diseases need to collaborate with each other alot more. This event went someway to facilitate this. Anyone involved in rare diseases would benefit for attendance at this annual conference. Details of future events can be found at https://camraredisease.org/

In loving memory of Daniel Harris, 1993-2019

We would to take this opportunity to remember what FD means to our families and community. We would like to take some time to think about the the loved ones we have lost to this heartbreaking condition, especially Daniel Harris, Age 26, who died in early October 2019 due to complications of Familial Dysautonomia - our sincere thoughts are with his parents and sister. We send thoughts and love to anyone who has lost someone from FD. If you are impacted from this story please know we are there to support you and can guide you to special counselling and advice.

Annual Supper Quiz 2019, raised £5,000

Thanks all your support at our annual supper quiz for FD-UK. We raised lots of funds, ate yummy food and even learned a little! Thanks to our quiz master, Michael Finger and all our

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Volunteers, our families and all our guests who made a difference by supporting the FD-UK community.

FD-UK Nominated Bryan King for a Lifetime Achievement in Volunteering Award (continued from p.1)

FD-UK is a small committee of which some members are parents of children with FD. Bryan has been supporting the committee members and their families since the creation of the Charity, and has not missed a monthly meeting in 40 years! Bryan has also brought in his family to assist with the running of this charity and its fundraising events. Together they have supported us to run annual events such as The Supper Quiz and Maccabi Fun Run. Without his support this charity would struggle to continue, as its members are often busy looking after their children and Bryan is always there to step in. Bryan has also helped to arrange for specialist Doctors from Israel to come to the UK to support families struggling with FD, and guide their physicians in the UK as this is such a rare condition. Bryan also oversaw the supply of lifesaving vests from the USA which assist in clearing the lungs and making the children more comfortable and able to breath more easily. Bryan is a backbone for the committee, with his experience of running the charity and its finances, bringing on new members and leading the way as the committee looks to its future and the challenges that brings. Bryan continues to help create events that engage those families and children with FD. His long-term enduring passion, commitment and achievements in helping others deal with this debilitating rare genetic condition deserves the recognition that this award would bring.

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