



# 4TH ANNUAL

## OPENMINDS GALA DINNER HELD ON MARCH 9TH, 2017 AT BIEL HALL 2.

Stylish geishas in a setting of cherry blossoms welcomed the guests. Over 600 prominent individuals from all walks of life came together to celebrate human diversity. It was a spellbound evening of Japanese elegance and cuisine coupled with Lebanese hospitality selflessly provided by artists and benevolent givers and emceed by the eloquent and handsome Wissam Hanna. The night included a rousing drummer intro, a popular medley of songs performed by the stunning Nancy Ajram, and inspiring music by pianist Guy Manoukian and his orchestra that transported our guests. Graceful and talented dancers from 'Dancing with the Stars' capped the entertainment for the evening. We continue to be humbled by the openhandedness and altruistic spirit of people in support of our cause! The gala's objectives are to provide OpenMinds with funds for vital research projects, education initiatives and training and medical assistance. Research progress, along with awareness, educational initiatives and state of the art medical care promotes locally relevant knowledge and positions the AUBMC Special Kids Clinic and the Neurogenetics Program at AUBMC as a center of excellence for autism and world class genetic research.



OpenMinds Gala 2017



Drummers

## INVESTING IN KIDS AND FAMILIES.....

### OPENMINDS PATIENT/FAMILY PACKAGES UPDATE:

It is only September and we have assisted over 237 families so far. In four years more than 1.2 million dollars have been set aside and are being spent to aid families in getting accurate diagnoses, genetic counseling/prenatal diagnosis, and to cover hospitalizations and game-changing early intervention for premature babies, children on the spectrum and others with neurogenetic and neurodevelopmental disorders.

### SECOND EPISODE... SHAHD KHODR, THE DAUGHTER OF ALI

A Syrian refugee from Idlib. She is a 3.5 year old girl with Hurler disease we introduced to you in our previous newsletter. Hurler syndrome causes facial deformities, stunted development and intellectual disability. After securing free enzyme replacement from the Genzyme/Sanofi compassionate program, which exceeds \$ 300 000/year, we took steps for a permanent solution, bone marrow transplantation from her brother. The cost for this exceeds \$55,000. A generous personal donation from Mrs. Ghida Rabbat propelled four other donors to come forth. Shahd is presently at AUBMC being conditioned for a bone marrow transplant (BMT) from her brother. To be continued.....



*Shahd & her brother      Shahd in the BMT unit*

## ‘CHILDREN CANNOT WAIT’

A roundtable discussion on the special needs of children with neurodevelopmental disabilities (ND) in times of conflict was held as part of the First Global Conflict Medicine Congress May 11th, 2017 at the Rafic Hariri School of Nursing. Speakers included UN Special Envoy Sigrid Kaag, Melissa Diamond from A Global Voice for Autism & Rita Flora Kevorkian from UNCHR and was attended by physicians, social workers, therapists and concerned caregivers.



*Sigrid Kaag speaking*

## RESEARCH PROGRESS

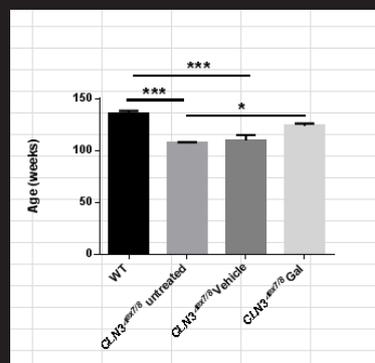
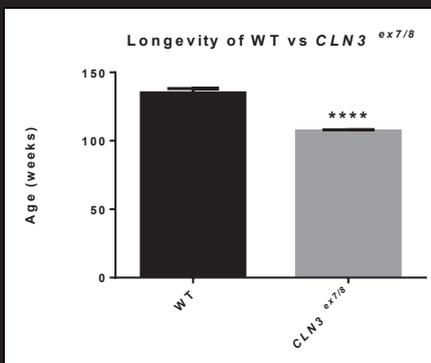
### AN UNUSUAL DIAGNOSIS MADE....BUT HOW DO YOU TREAT IT?

Many physician-scientists strive to address this through applied research. In this age of genetics and genomics we in Lebanon have to pull our own weight and contribute to medical and scientific progress. Support for research by our visionary OpenMinds board, made possible by the generosity of our donors has made this a reality. Work on the novel treatment, galactosylceramide, specific for the rare genetic disorder, CLN3 disease, has been licensed to SENEBA, Inc. PhD student Sally El-Sitt had her work on a CLN3 mouse model presented both in Boston at the international NCL meeting and at the NCL-Stiftung in Hamburg.



Our Sally El-Sitt in Hamburg at the NCL-Stiftung

### Longevity



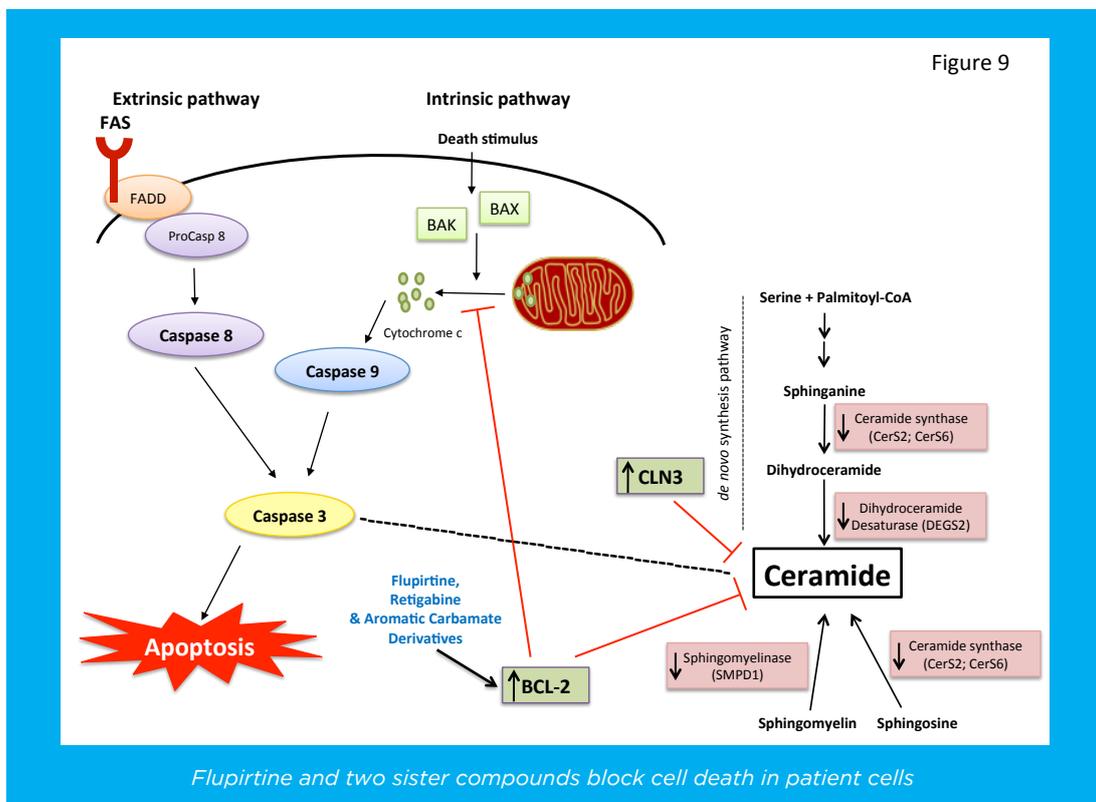
**Life span:**

- 135 weeks +/- 3.2 weeks for WT mice
- 107 weeks +/- 0.8 weeks for CLN3<sup>ex7/8</sup> mice

Improved longevity in CLN3 mice treated with GalCer

Three novel compounds tested on cell lines derived from kids with the deadly neurogenetic disorders CLN1/2/3/5/6/8 showed effectiveness in preventing cell death and lowering the levels of toxic ceramide in them. CLN2 and CLN6 disease occur in Lebanon. The ASKC clinic gets numerous referrals of kids with CLN3, CLN2 and CLN6 disease from Syria, Iraq, Kuwait and the Ukraine.

Fadi Saadeh, an AUBMC 4th year medical student presented this work at the International NCL conference in Boston, and received a \$ 1000 award from the Diana Tamari Sabbagh foundation for best scientific research for 2017. Based on this work a new patent application has just been submitted entitled: Functionalized Pyridine Carbamates with Enhanced Neuroprotective Activity. Inventors: Paul Trippier, Nihar Kinarivala, Rose-Mary Boustany (Texas Tech University and AUB). Application number 62/532,624. Filing date: 7/14/2017.



## PUBLICATIONS:

1. Silva Kourtian, Jihane Soueid, Nadine J. Makhoul, Dikran Richard Guisso, Maria Chahrour, and Rose-Mary N. Boustany. **Candidate Genes for Inherited Autism Susceptibility in the Lebanese Population.** Scientific Reports. 2017; 7: 45336, 2017 Mar 30. doi: 10.1038/srep45336
2. Dahlia Saab, Monique Chaaya, Rose-Mary Boustany. **Prevalence and correlates of Autism Spectrum Disorder: Lebanese cross-sectional study (BMC Pediatrics BPED-D-17-00353, 2017 in review).**
3. Dikran Richard Guisso, Fadi S. Saadeh, Dahlia Saab (MPH), Joud El Deek, Sarah Chamseddine, Hadi Abou El Hassan, Ghidaa Majari, Rose-Mary Boustany. **Association of autism with maternal infections, perinatal and other risk factors: a case-control study (JADD, in review 2017).**
4. Ghazale H, Ramadan N, Mantash S, Zibara K, El-Sitt S Darwish H, Chamaa F, Boustany R-M, Mondello S, Abou-Kheir W, Soueid J, Kobeissi F. **Docosahexanoic acid enhances the therapeutic potential of neonatal neural stem cells transplantation post-traumatic brain injury (in review Scientific Reports, 2017).**
5. Nihar Kinarivala, Ronak Patel, Rose-Mary Boustany, Abraham Al-Ahmad and Paul C. Trippier. **Discovery of Aromatic Carbamates that Confer Neuroprotective Activity by Enhancing Autophagy and Inducing the Anti-Apoptotic Protein Bcl-2 (Journal of Medicinal Chemistry, 2017 in review: jm-2017-011994).**
6. Shbarou Rolla M, Boustany Rose-Mary, Daher Rose T, Pakdel Parisa El Eid Rozana, Nouredine Abir, Karam Pascale E. **Non ketotic hyperglycinemia: 14-year experience in Lebanon.** (in review, Pediatric Neurology, 2017).
7. Sally El-Sitt, Jihane Soueid, Jamal Al-Ali, Joelle Makoukji, Nadine Makhoul, Hayat Harati, Rose-Mary Boustany. **Elevated ceramide levels in CLN3 deficient patients and Cln3 ex7/8 mice and a developmental comparison between wild type and Cln3 ex7/8 mice sera and brain (submitted, Neurobiology of Disease 2017)**

## INTEGRATING LIFE AND LEARNING...

### “SPREAD THE BLUE LOVE”- WORLD AUTISM MONTH

We lit it up blue at the ASKC clinic in the Pierre Abu-Khater building on April 3 for World Autism Awareness Day. The campaign was organized by OpenMinds and the AUBMC Special Kids Clinic (ASKC) in conjunction with the ADrising team with kids, their siblings, parents, friends and staff. The building shimmered blue every night for a week. The event was immortalized with blue paint hand prints on a white canvas that now has pride of place in the clinic! The evening ended with merriment, blue cupcakes, lively music and the release of 100 Blue Balloons into the cool Beirut evening sky.



Children, therapists and parents releasing balloons into the sky

Blue paint handprints!

### WORKSHOP ‘UNIVERSAL DESIGN FOR LEARNING’ AT THE ANTONINE INTERNATIONAL SCHOOL

Last year OpenMinds sent a team of professionals, board members and parents to Harvard to attend a program entitled “**Universal Design for Learning: Leading Inclusive Education for All Students**” (UDL). This knowledge was put into practice by hosting a two day workshop on UDL at Antonine International School (AIS). The workshop was given by Mrs. Lara el Khatib in collaboration with OpenMinds to a group of teachers at Antonine International School in March of this year. They were taught key principles of UDL by analyzing concrete examples and real life case studies. At the end of the workshop, teachers evaluated its quality and content.



UDL Workshop at AIS

## IT TAKES A VILLAGE... OPENMINDS DIBBYEH PROJECT:

Thanks to Dar Group and ProFinance, and the hard work and ideas of dedicated board members, a conceptual design, as well as a proposed feasibility plan were finalized this past spring.



## FUNDRAISING DINNER WITH PURPLE CAPITAL GROUP

Purple Capital Group organized a Fundraising Dinner in Monaco. All proceeds were donated to OpenMinds this year. The event was held at the Yacht Club of Monaco on the 20th of May where 200 guests from the international community and Lebanon attended the dinner, dancing away the night to dynamic live music, amidst entertainers and a tombola that kept on giving!! We had generous donors that evening, most notably Bob and Tamar Manoukian.



## A SPECIAL SHOUT OUT TO OUR

'Pinnacle & Prized Partners' for their constant support:

## THANK YOU

### Pinnacle Partners



CAPITAL GUIDANCE

Mr. & Mrs. Bob Manoukian



### Prized Partners

