6th Annual Summit on Rare Diseases and Orphan Drugs

12th International Conference on Bacteriology and Infectious Diseases

JUNE 23, 2022 | WEBINAR

PLGA nano carrier for targeted delivery of drugs in neuroinflammation

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 \mathbf{N} anoscience and nanotechnology have shown unparalled growth in research and applications in recent years. There is growing hope that nanotechnology, when applied to medicine, will lead to significant advancements in disease diagnosis and treatment. Drug delivery, both in vitro and in vivo diagnostics, nutraceuticals, elicits development of more biocompatible materials for use in medicinal field.

Neuroinflammation, the response of the central nervous system (CNS) to disturbed homeostasis, typifies all neurological diseases, including developmental, traumatic, ischemic, neoplastic, infectious and <u>neurodegenerative disorders</u>. From several vantage point, the brain is the most arduous organ for delivering drugs. First, as the population ages, the prevalence of degenerative brain illnesses will rise. Second, the bloodbrain barrier (BBB) is well-known as the body's finest drug

gatekeeper against the exogenic substances. Dexamethasone is known to inhibit inflammatory response, the severe side effects associated with high dose of glucocorticoids required to reach therapeutic value, is one of the main reasons for not using dexamethasone as a neuroprotective agent. Nanotechnology offers a suitable alternative route in drug delivery. In particular, the rationale of using nanoparticle (NPs) for brain drug delivery may promote their targeting of the BBB and the enhancement of its crossing. Poly lacticco-glycolic acid (PLGA) is the most studied and best defined polymer, approved by the Food and Drug Administration of USA (FDA) for drug delivery and pharmacological studies. In this study we attempted to determine the most efficient method for the synthesis of doped PLGA nanoparticles for drug delivery applications and varying the type of surfactants used and its role in drug delivery.

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<u>Clinical diagnosis of lambert-eaton myasthenic syndrome: The power of taking time with</u> <u>patients</u>

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ambert-Eaton myasthenic syndrome (LEMS) is an uncommon disorder of neuromuscular junction transmission. The predominant clinical manifestation is slowly progressive muscle weakness particularly involving the legs, depressed or absent deep tendon reflexes, and ocular symptoms. The incidence of <u>LEMS</u> is classified as rare, the prevalence unknown.

This case focuses on a young female presenting with vague symptoms given the presumptive diagnoses of more common

causes. <u>Anamnesis</u> revealed she is following the clinical trajectory of several family members of different generations who died in their midlives.

By taking the time to document a thorough sequence of clinical events and family history, proper confirmatory tests can prompt further work-up to ascertain underlying malignancies that can be treated early, gaining the patient years of quality life and a sense of peace by knowing what is happening with their health.

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<u>The impact of covid-19 on patients and families with rare and intractable diseases</u> (NANBYO) in Japan - From the perspective of healthcare provisio

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We conducted the following two surveys of patients and families living in Japan with rare/intractable diseases (NANBYO) under the covid-19 outbreak.

- Quantitative study: To clarify the impact of covid-19 on the lives and medical situations of patients and families with NANBYO, the survey was conducted at a point in time by adding some original items to the items in the EURORDIS survey. The survey was conducted between May and October 2020, with 363 valid responses.
- Qualitative study: To analyse narrative data related to covid-19, we asked 110 patients/families to complete an open-ended questionnaire once a month for nine months, from May 2020 to January 2021.

Results of study (1) showed that 90% of NANBYO patients recognized covid-19 as a threat to their own lives. It was "very threatening," especially for patients with renal/urologic, immunologic, and <u>cardiovascular diseases</u>. In addition, 37% of all patients had experienced treatment interruption or postponement. 29% of all respondents had experienced telemedicine (including telephone medical care). Of those,

98% rated the online consultation as helpful. Patients also commented that the ability to provide telemedicine and <u>drug delivery</u> has improved convenience for patients living in remote areas.

This presentation will include a report on the disruption of drug distribution and medical care in Japan because of this outcome and individual cases of patients who were forced to discontinue treatment.



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