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The Cholangiocarcinoma
Foundation



www.cholangiocarcinoma.org

Specific Aim

- Patient perspective
- Cholangiocarcinoma resources for patients and physicians/researchers
- Multi-institutional national/international clinical trials



Patient Perspective

- Patient preference for care reflect their values, understanding or misunderstanding of their illness,
- Rare diseases fundamentally lack resources for disease specific information
- Support systems
- Specialists for treatment of CCA
- Knowledge of major cancer centers focused on rare diseases



Patient Understanding of Prognosis is Imperfect

- Tend to overestimate probability of long term survival
- Faced with a fundamental choice between cancer-directed therapies to control the underlying disease with possible life extending therapy but with associated risks and benefits of treatment choice vs. palliative/comfort care with emphasis on symptom management



CCF Critical Component in Patient Education

- Empowers patients to engage with specialists in order make educated decisions
- Provides computer based support
- Internet health web links, brochures and other downloadable information
- Global Discussion Board – uncensored but moderated



Clinical Trials

- CCF help define and aid in the development of strategies to increase patient recruitment, compliance and retention
- Engage with Advocates in early phase of study design
- Although individual data can't be shared – aggregate data can be provided to participants



Partnering with CCF

- PI initiated studies provided in patient friendly version – 8th grade reading level
- CCA provides questionnaire of simplified study protocol:
- Study Center
- Study Contact
- Basic Study Overview
- Projected Patient Enrollment
- Study Start and Completion Date
- Inclusion/Exclusion Criteria
- Required Pre-study Tests
- Potential Side Effect Treatment



CCF Focus on Providing the Link

- Between patients Community Oncologist and Clinical Research Studies
 - Patients can Copy and print information from our website – to discuss with treating physician



Partnering with CCF

- Aid in establishing multi-institutional trials within US and abroad
- Help establish a paradigm in rare tumors
- Each country performing an in-country protocol with identical eligibility and endpoints
- Followed by pre-planned combined analysis



Suggestions

- Trials performed in parallel would avoid many administrative/regulatory issues
- Decrease the power of the individual studies more consistent with phase II, but when analyzed together, they achieve power more in line with phase III
- Individual studies that target an endpoint that can be reached with fewer patients in a given time frame, for example targeting PFS in individual studies, but combining them to look at OS



Recommendations

- Target an endpoint in individual studies
- Endpoint with fewer patients
- Set time frame
- Example targeting PFS in individual studies
- ... but combining them to look at OS



In Curative Setting

- **PERCUTANEOUS BIOPSIES AND/OR BIOPSIES PERFORMED AT THE TIME OF ENDOSCOPIC ULTRASOUND ARE DISCOURAGED**
- Based on Mayo Study Endoscopic Ultrasound (EUS) – guided acquisition is fraught in an inappropriately high rate of needle tract tumor seeding.



Razumilava, N, Gores, GJ. Cholangiocarcinoma Lancet 2014;383:2168-79

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Newly Diagnosed

- Liver Transplantation/Perihilar CCA:
- [The American Association for the Study of Liver disease Guidelines recommends](#) that potential transplant candidates should be expeditiously referred to [major cancer centers](#) that have established protocols for oncologic assessment and treatment approved by [United Network for Organ Sharing \(UNOS\)](#).



Proposed Initiatives

- Physician Portal
- Medical Advisory Board approved participation
- Forum for clinical exchange - i.e. case reports
- Interact with global investigators



Thank you



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